

SICKLE CELL DISEASE

Up Close and Personal

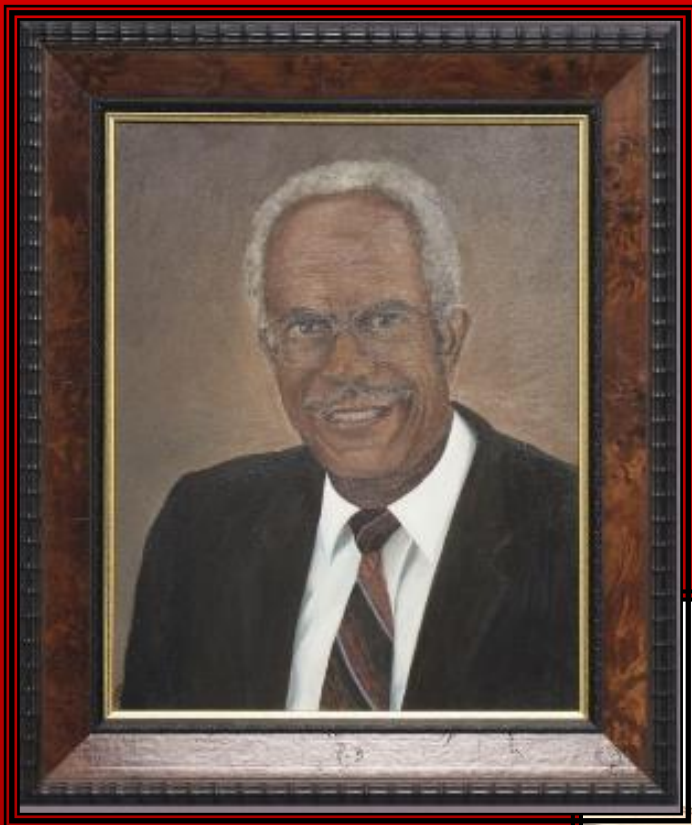
Wanda Whitten- Shurney MD
Sickle Cell Association of America
Michigan Chapter
shurneyw@scdaami.org

Disclosure



OUTLINE

- SCDAAMI
- History
- Sickle Cell Disease in General
- Challenges Patients Face
- Good News





OUR MISSION

- TO MAXIMIZE THE QUALITY LIFE OF INDIVIDUALS LIVING WITH SCD
- TO ENABLE INDIVIDUALS WITH SICKLE CELL TRAIT TO MAKE INFORMED DECISIONS THAT THEY BELIEVE ARE IN THEIR BEST INTEREST WITH RESPECT TO FAMILY PLANNING
- TO PROVIDE EDUCATION, TESTING AND INFORMATION FOR THE GENERAL PUBLIC
- SERVE AS THE COODINATING CENTER FOR THE NEWBORN HEMOGLOBINOPATHY SCREENING PROGRAM FOR THE MDHHS

SCDAA MI Patient Advocates

Ann Arbor
Benton Harbor
Detroit (CHM/PVT)
Flint
Grand Rapids
Jackson
Kalamazoo
Lansing
Muskegon
Pontiac
Saginaw



STATEWIDE PROGRAM

- DISEASE EDUCATION
- CARE COORDINATION
- SUPPORT SERVICES
 - Medical referrals
 - SSI/SSD
 - IEP/504 plans
 - Basic needs
 - Career Counseling
- SUMMER CAMP PROGRAM
- SPECIAL ACTIVITIES

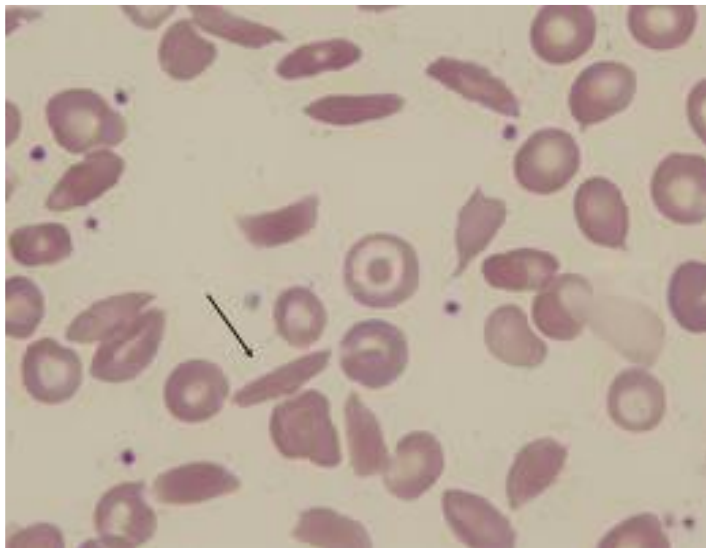
A 3D illustration of a blood vessel, showing a cross-section of the vessel wall and numerous red blood cells (erythrocytes) flowing through the lumen. The red blood cells are depicted as biconcave discs, some in sharp focus and others blurred to suggest motion. The overall color scheme is dominated by deep reds and dark reds, with the word "HISTORY" overlaid in a bold, white, sans-serif font in the center.

HISTORY



James Herrick – 1910

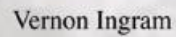
First patient diagnosed
With SCD in United States



Walter Clement Noel



*"peculiar elongated and sickle shaped"
red blood cells in
"an **intelligent** negro of 20."*





**Chwechweechwe
(Ga)**

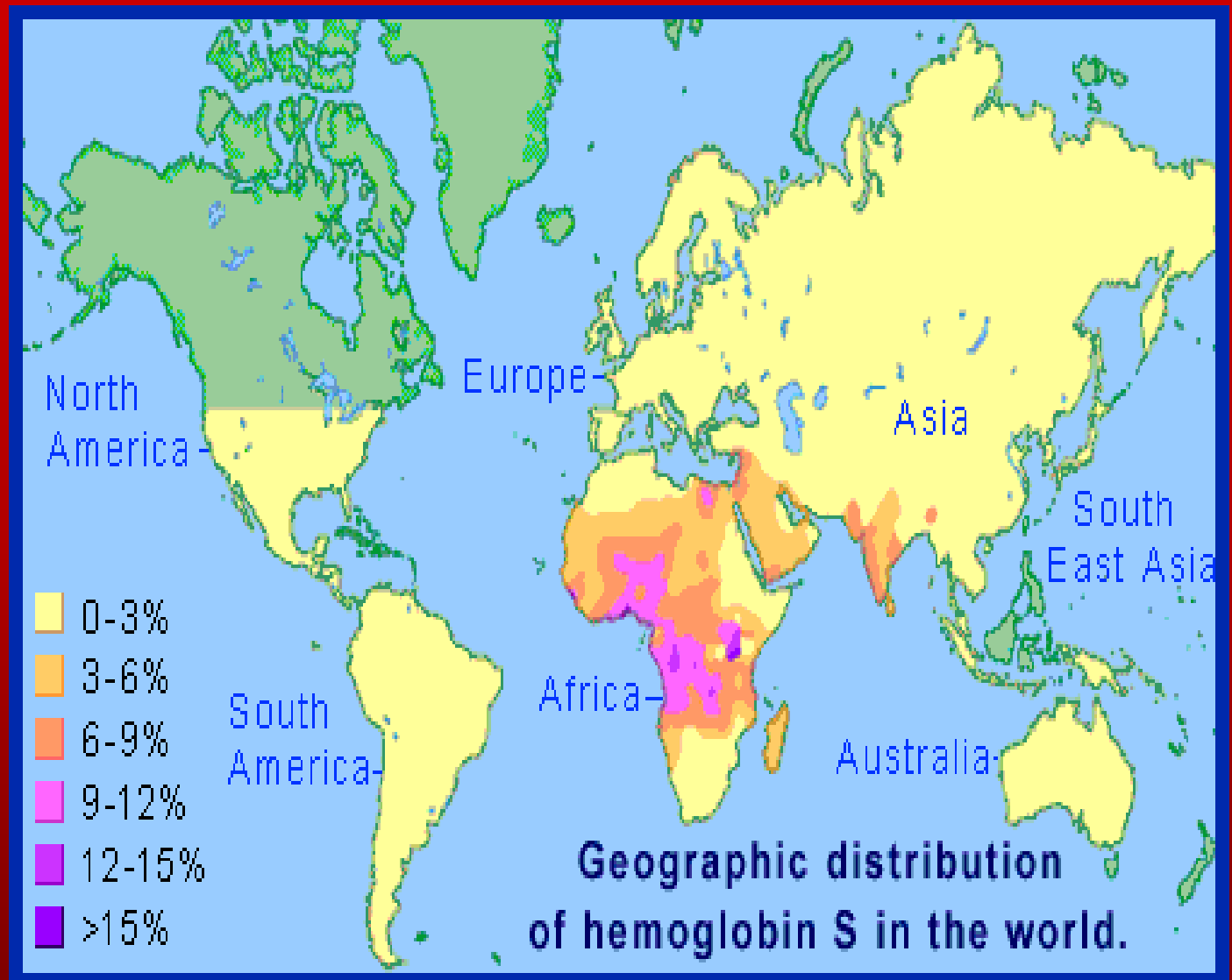
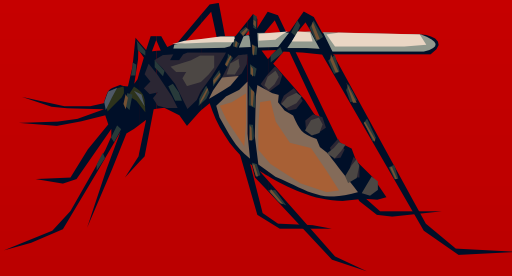
Nwiiwii (Faute)



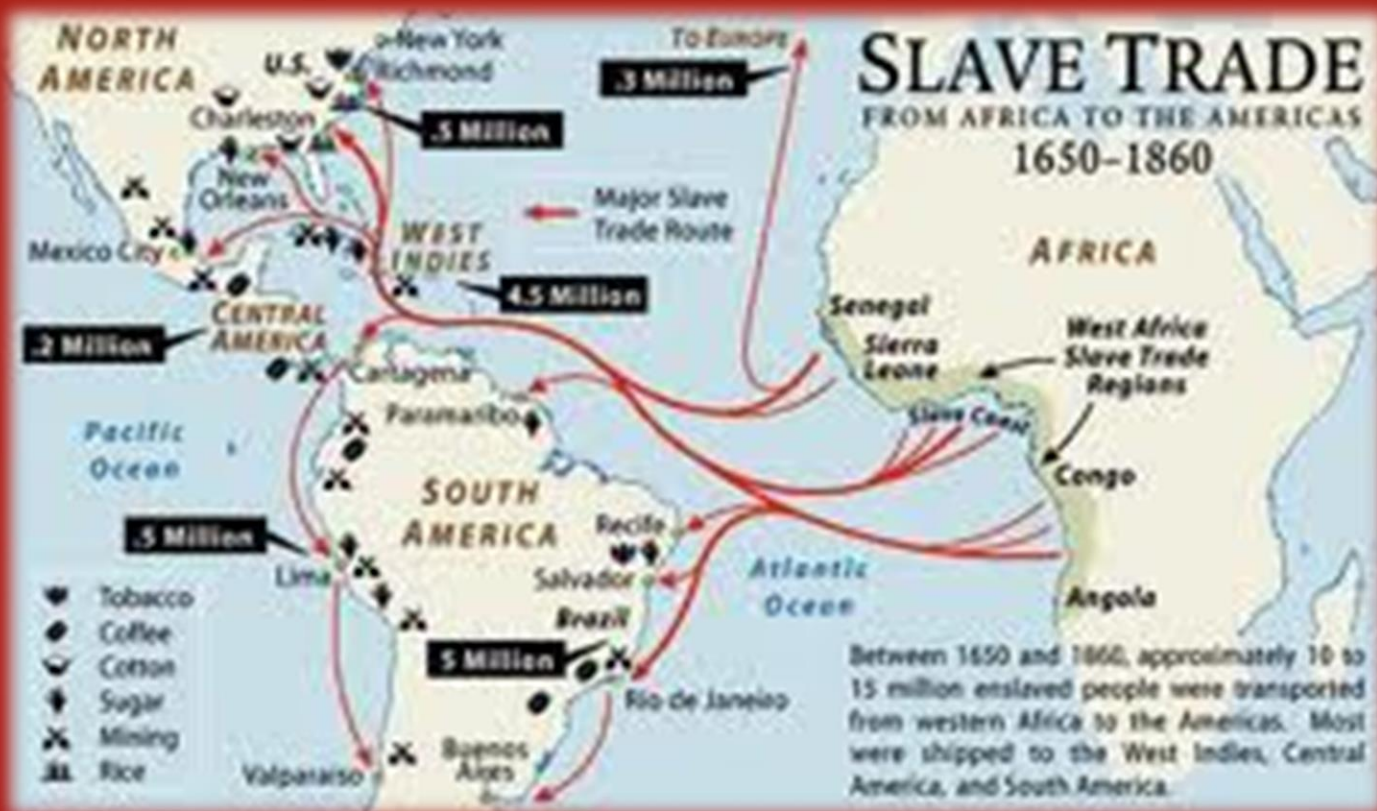
Ahotutuo (Twi)

Nuidudui (Ewe)





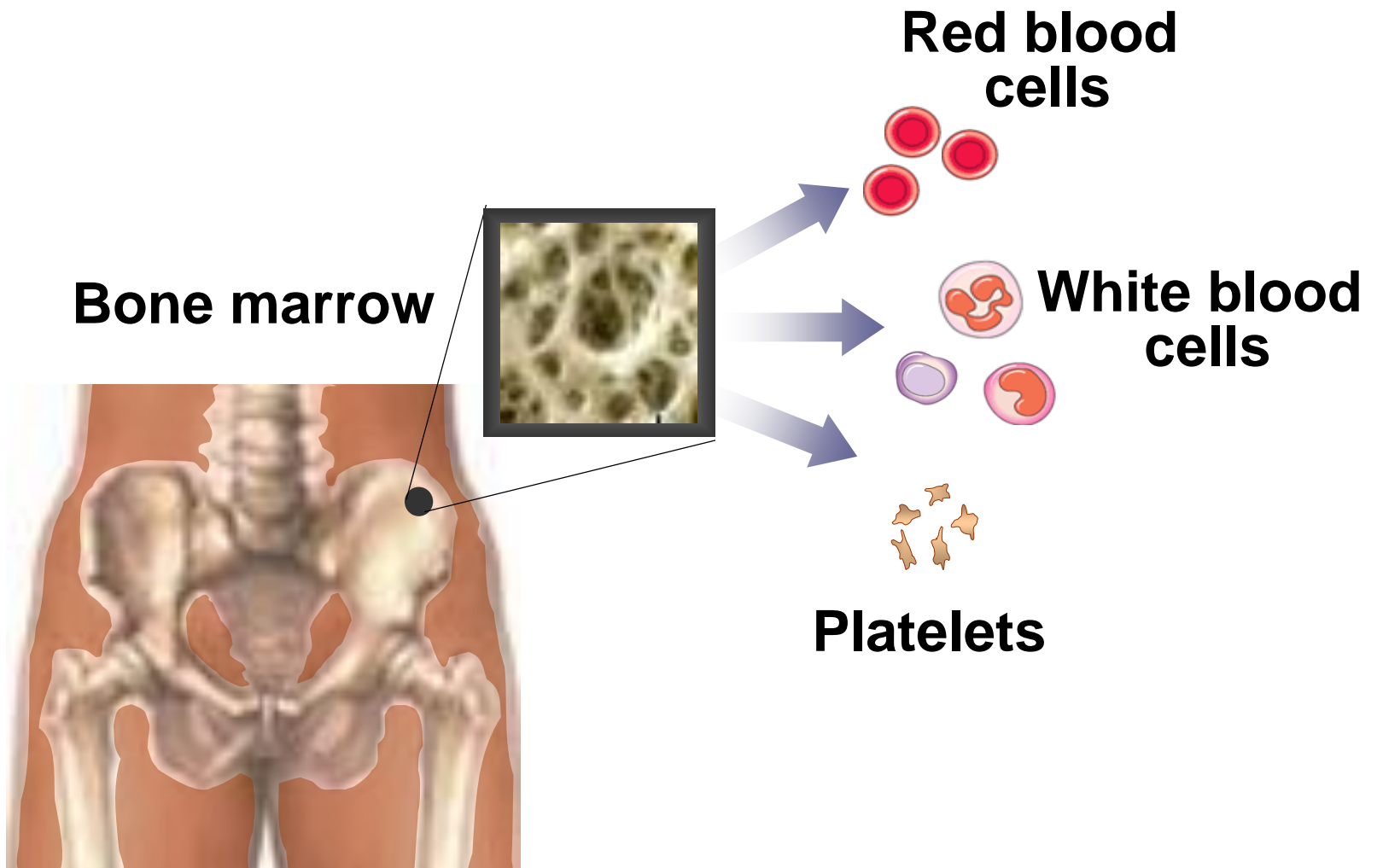
MALARIA

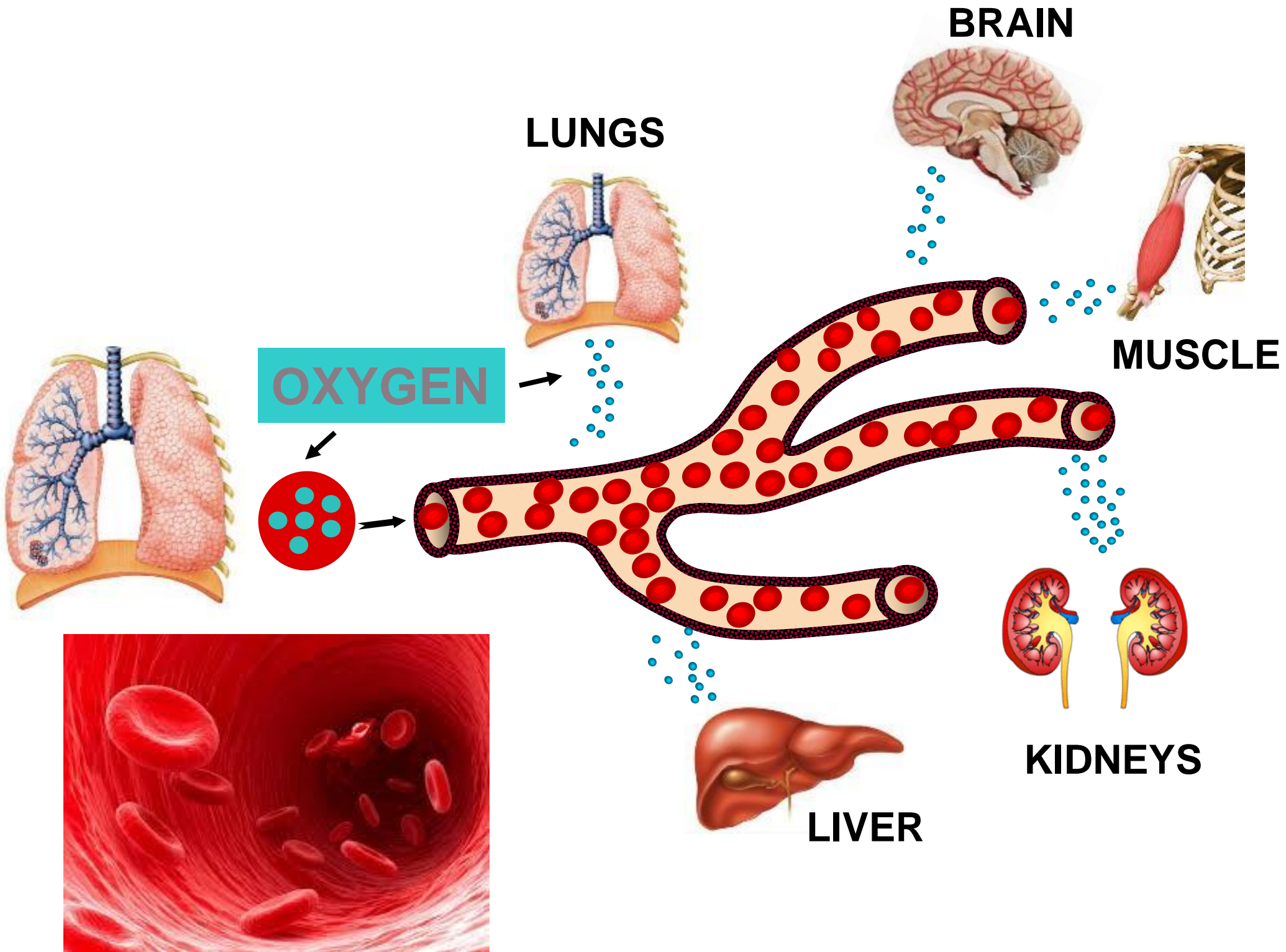


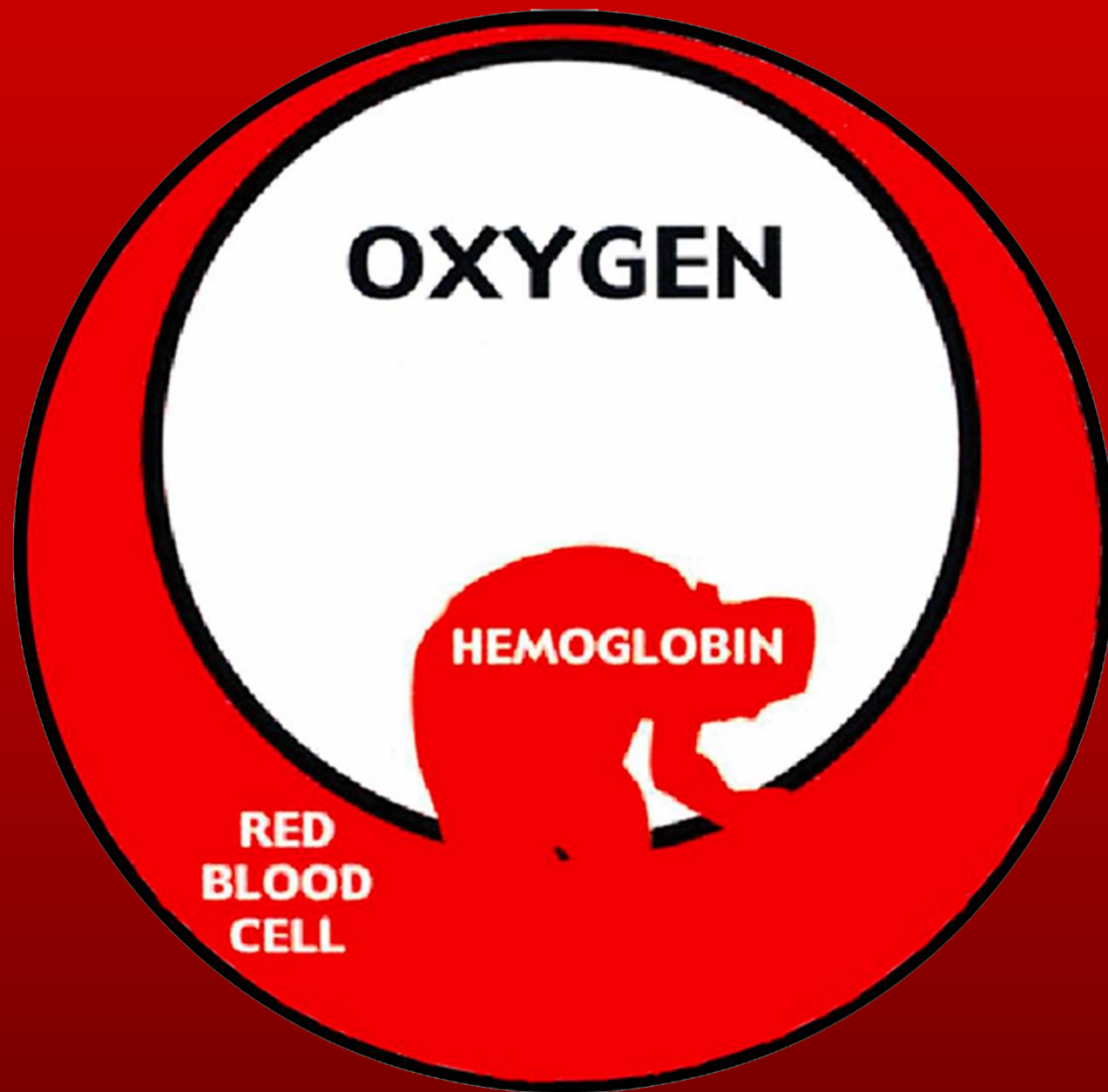




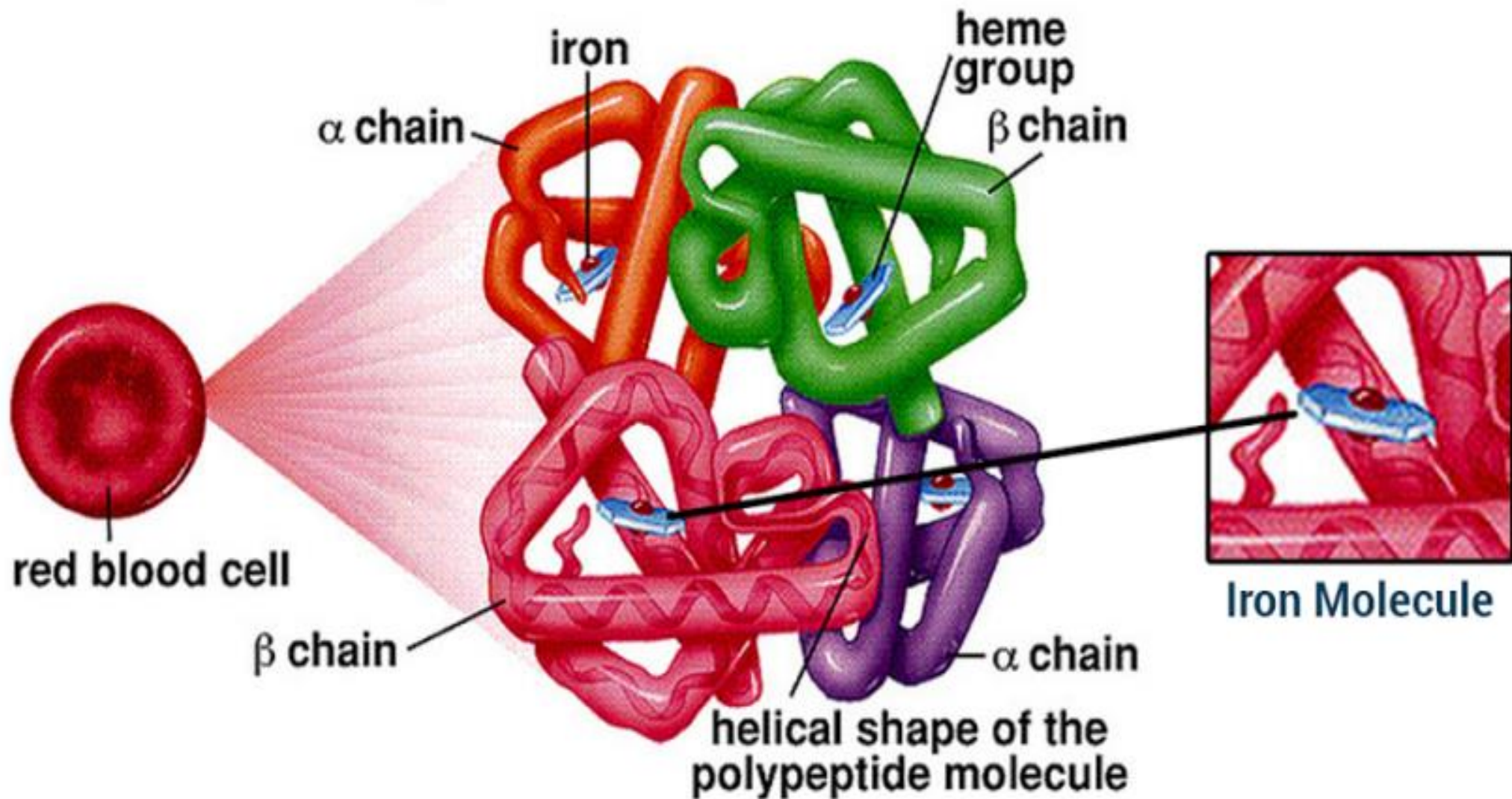
WHAT IS SCD







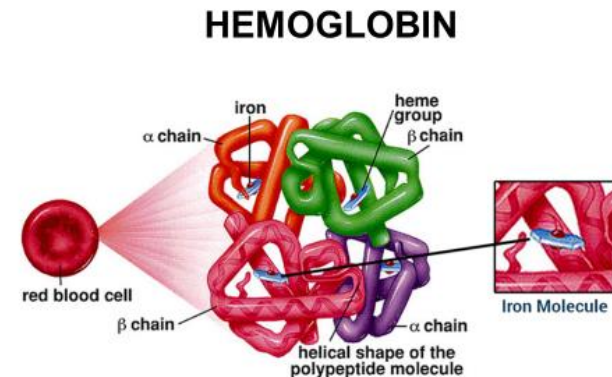
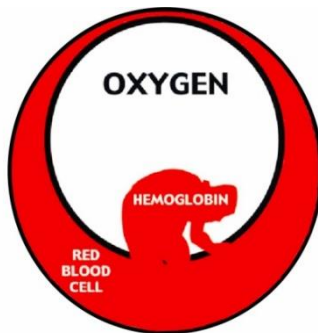
HEMOGLOBIN

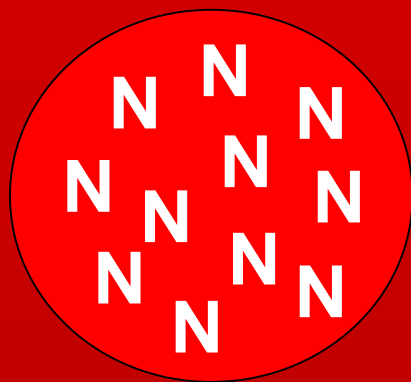


**“NORMAL” ADULT HEMOGLOBIN
HEMOGLOBIN A**

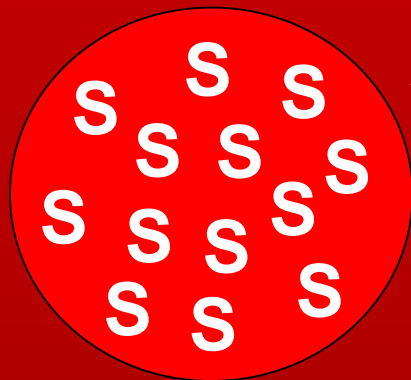
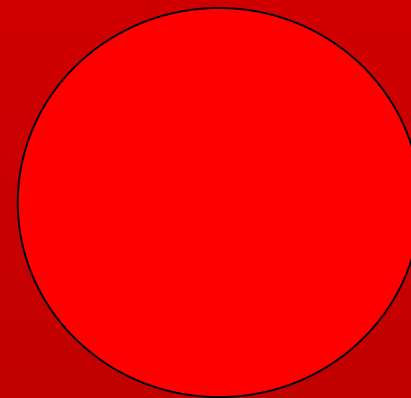
**SICKLE HEMOGLOBIN
HEMOGLOBIN S**

HEMOGLOBIN C

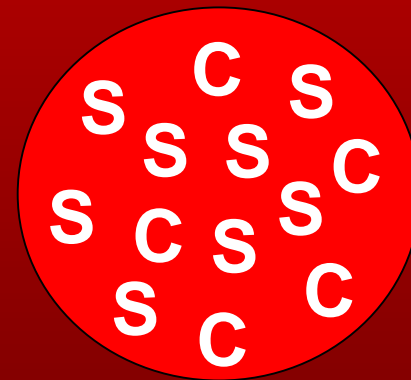
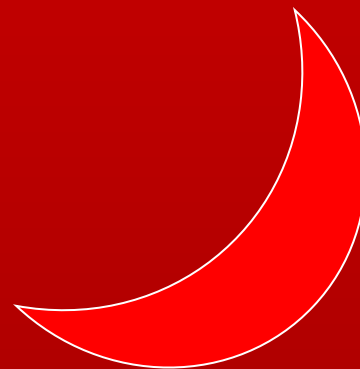




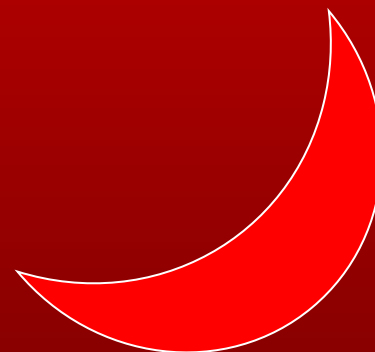
O_2



O_2



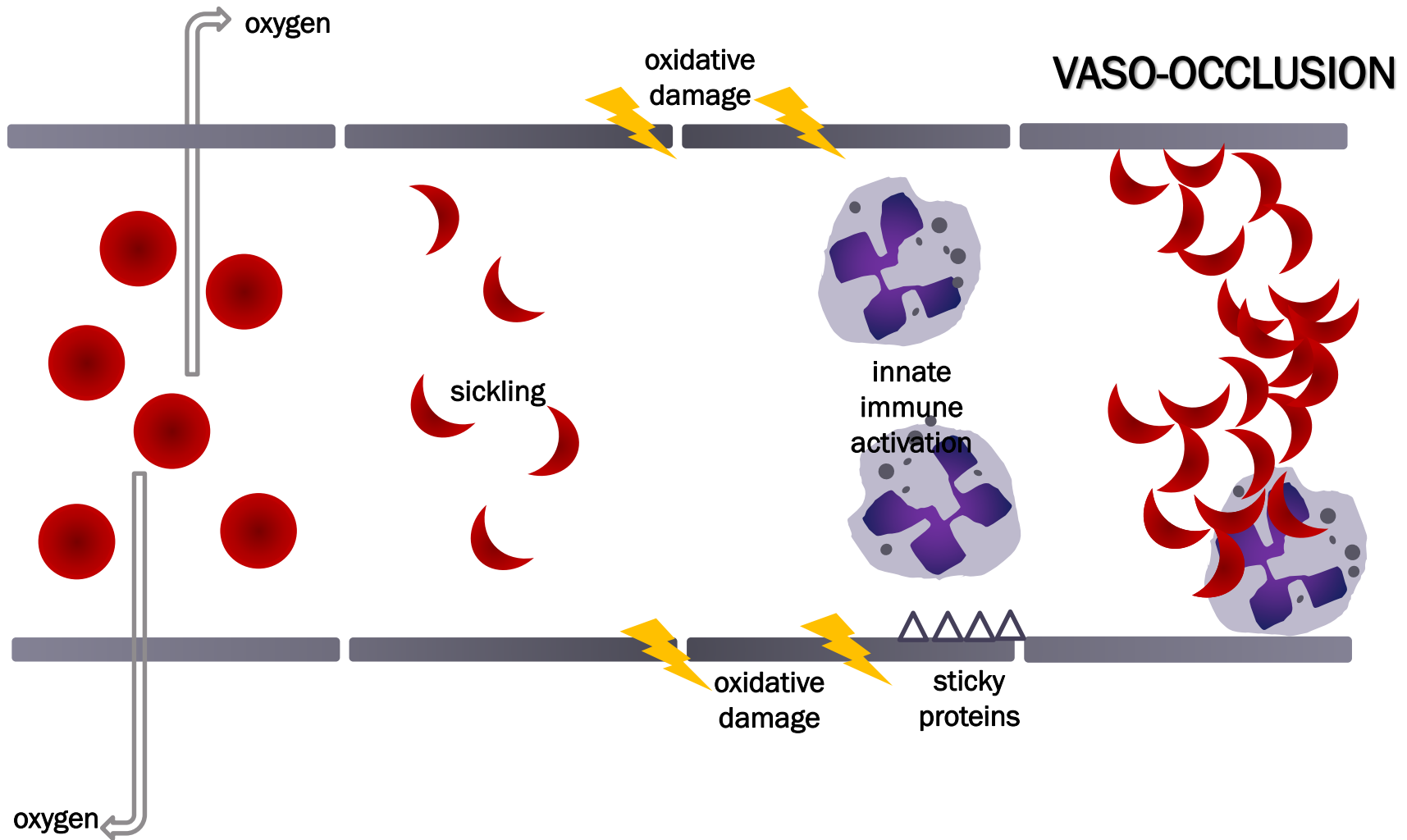
O_2



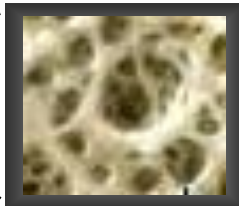
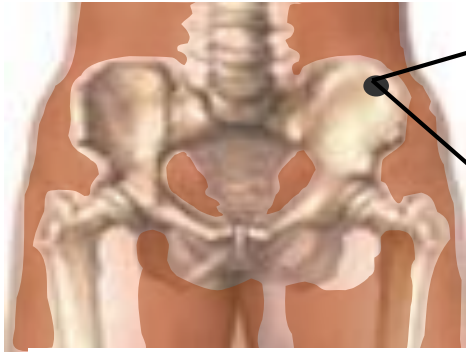


A microscopic image of a blood vessel, likely a capillary or small vein, showing a dense collection of red blood cells. The vessel is filled with these cells, which appear as small, reddish-brown discs. The vessel wall is visible as a thin, translucent layer. The background is a blurred mix of pink and purple hues, suggesting a tissue environment. Overlaid on the right side of the image is the text 'Pain', 'Organ', and 'Damage' in a black, italicized font.

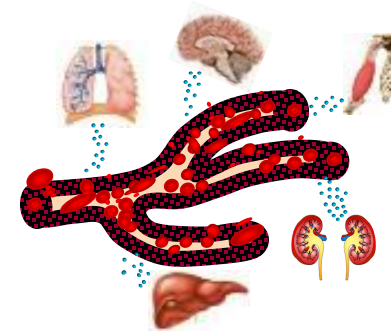
Pain
Organ
Damage



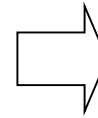
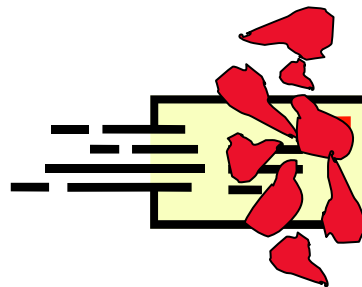
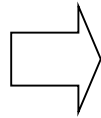
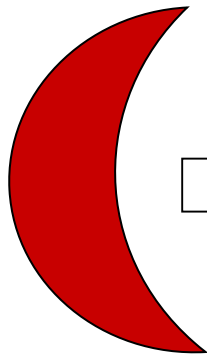
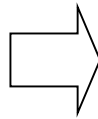
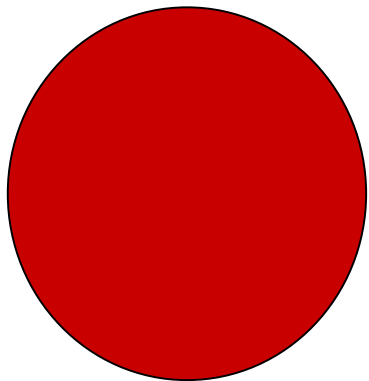
BONE MARROW



**RED BLOOD
CELLS**



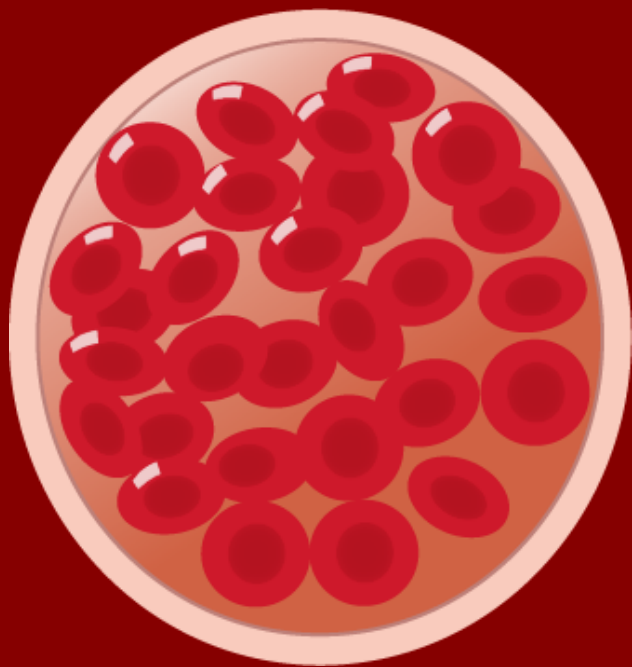
CIRCULATION



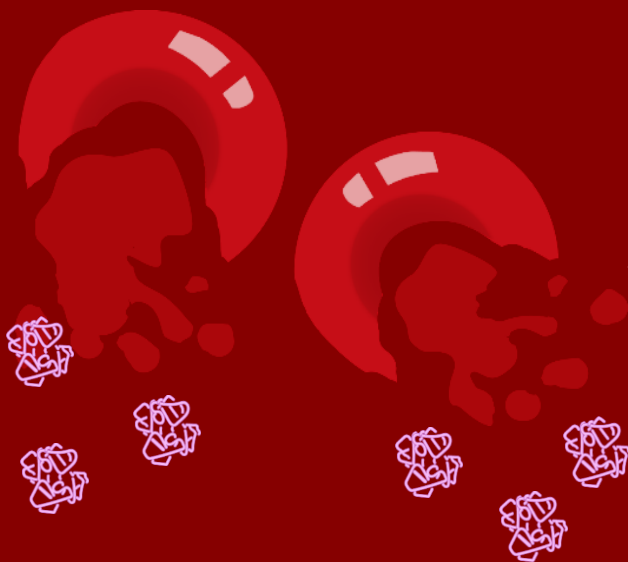
ANEMIA



FATIGUE



Hemoglobin



Spillage of contents

BLOCKAGE

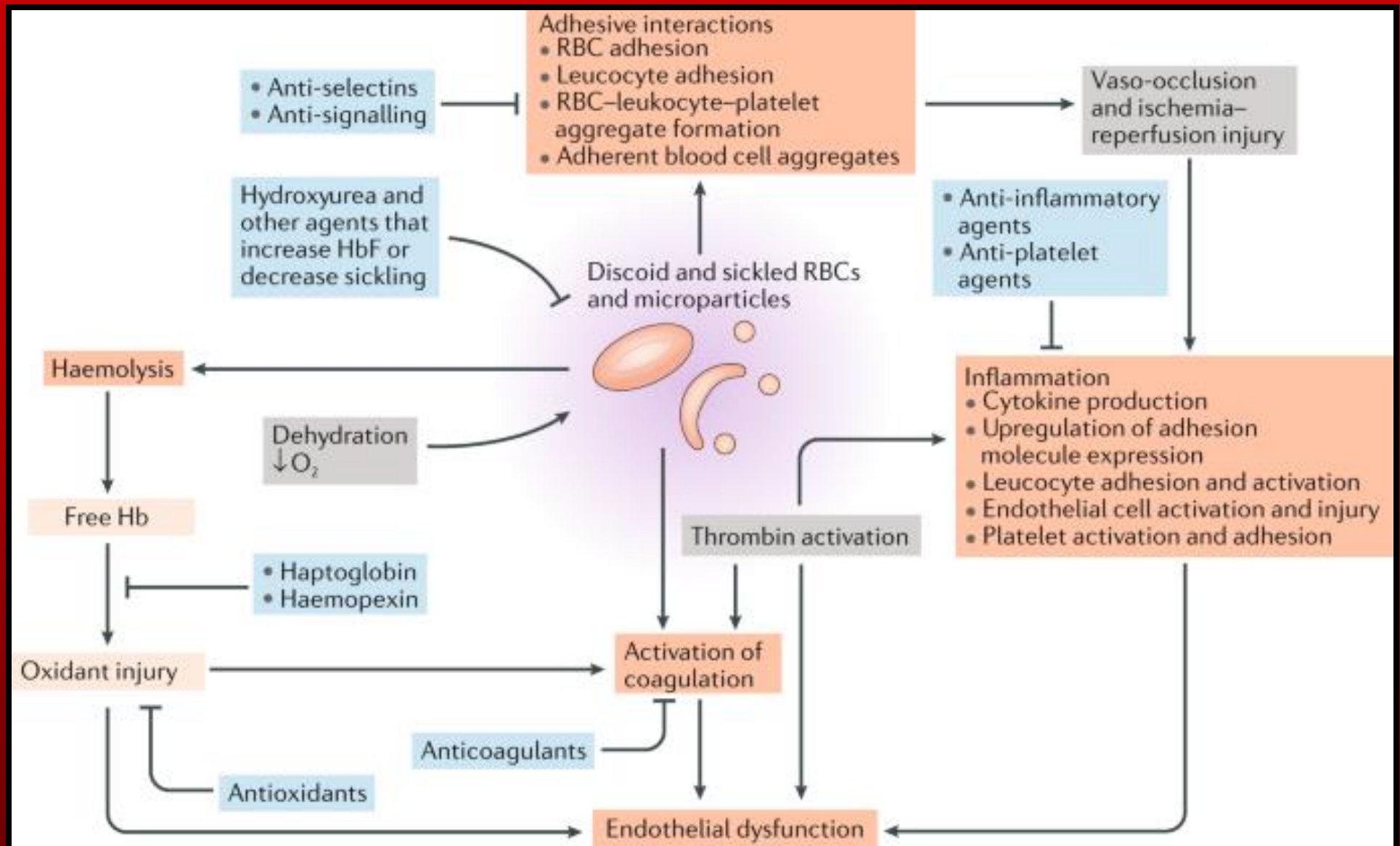
(VASOOCCLUSION)

&

BREAKDOWN

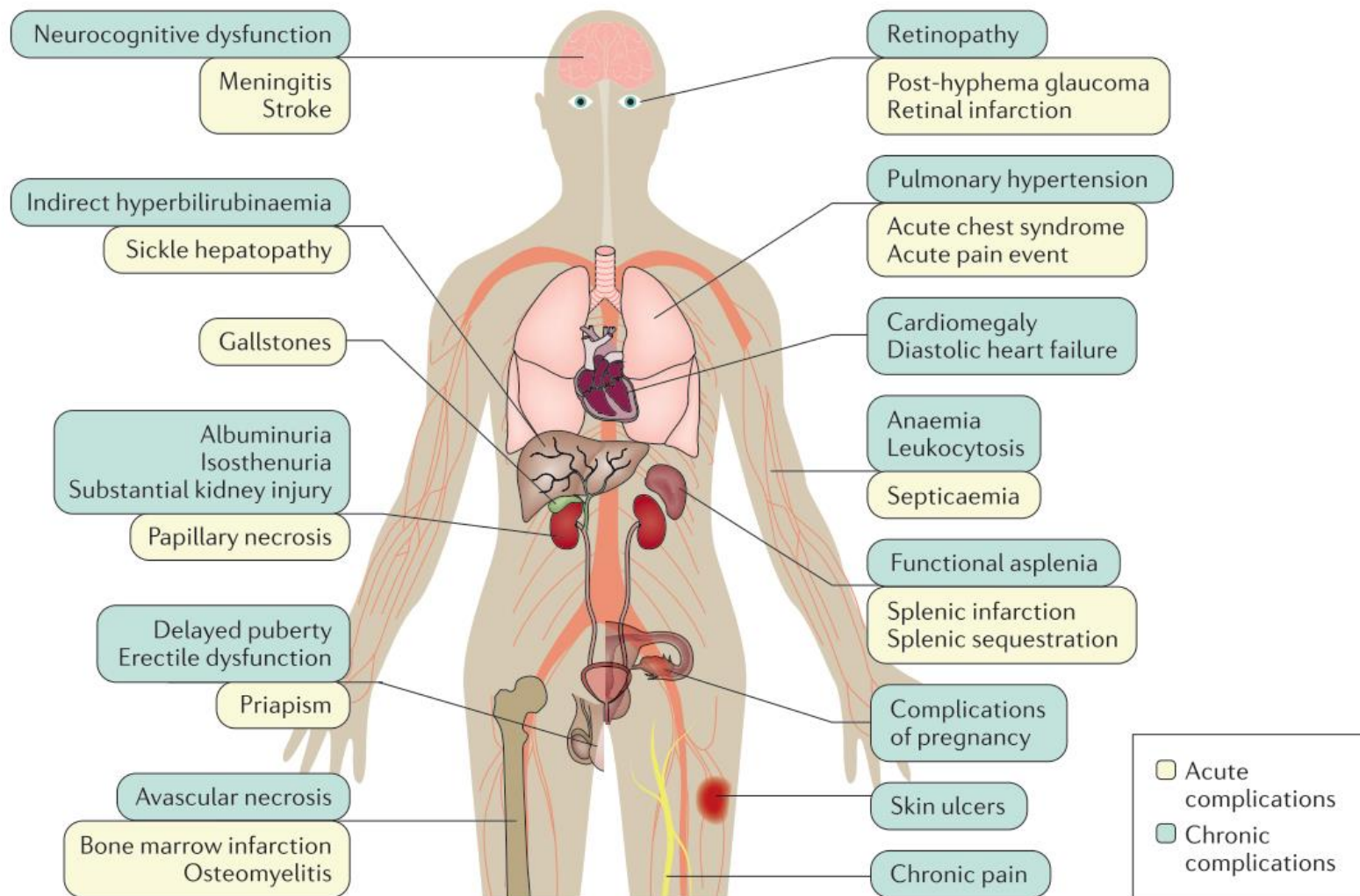
(HEMOLYSIS)

COMPLEX PATHOPHYSIOLOGY





HEALTH PROBLEMS

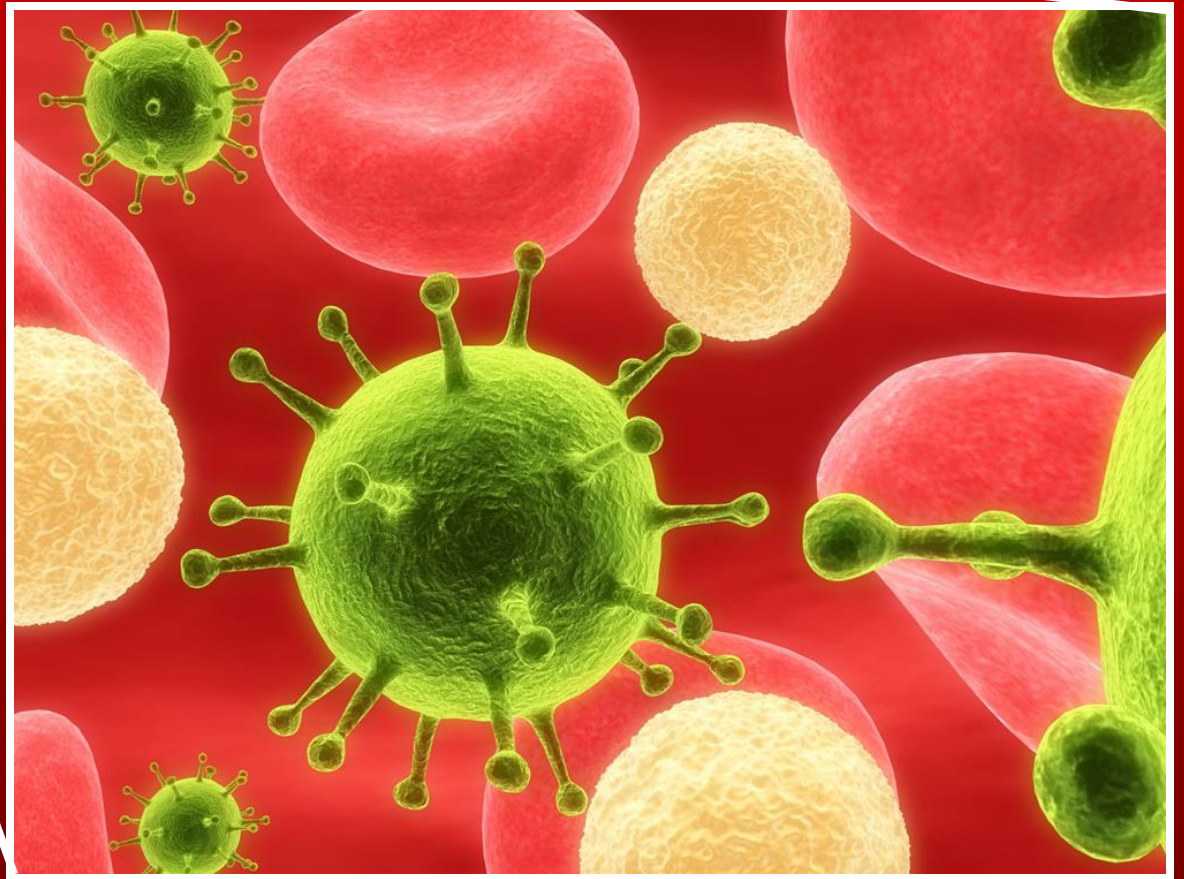


Phenotypic Heterogeneity





**STREP
PNEUMO**



BACTERIA



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JUNE 19, 1986

Number 25

PROPHYLAXIS WITH ORAL PENICILLIN IN CHILDREN WITH SICKLE CELL ANEMIA

A Randomized Trial

MARILYN H. GASTON, M.D., JOEL I. VERTER, Ph.D., GERALD WOODS, M.D., CHARLES PEGELOW, M.D.,
JOHN KELLEHER, M.D., GERALD PRESBURY, M.D., HAROLD ZARKOWSKY, M.D., ELLIOTT VICHINSKY, M.D.,
RATHI IYER, M.D., JEFFREY S. LOBEL, M.D., STEVEN DIAMOND, M.D., C. TATE HOLBROOK, M.D.,
FRANCES M. GILL, M.D., KIM RITCHEY, M.D., AND JOHN M. FALLETTA, M.D.,
FOR THE PROPHYLACTIC PENICILLIN STUDY GROUP

Gaston et.al.



Routine Immunizations:

Prevnar 20

Meningococcal

Influenza

COVID 19



Name _____

Type of sickle cell diseases

_____ SS
 _____ SC
 _____ S Thal Zero
 _____ S Thal Plus

Usual Hemoglobin Level _____



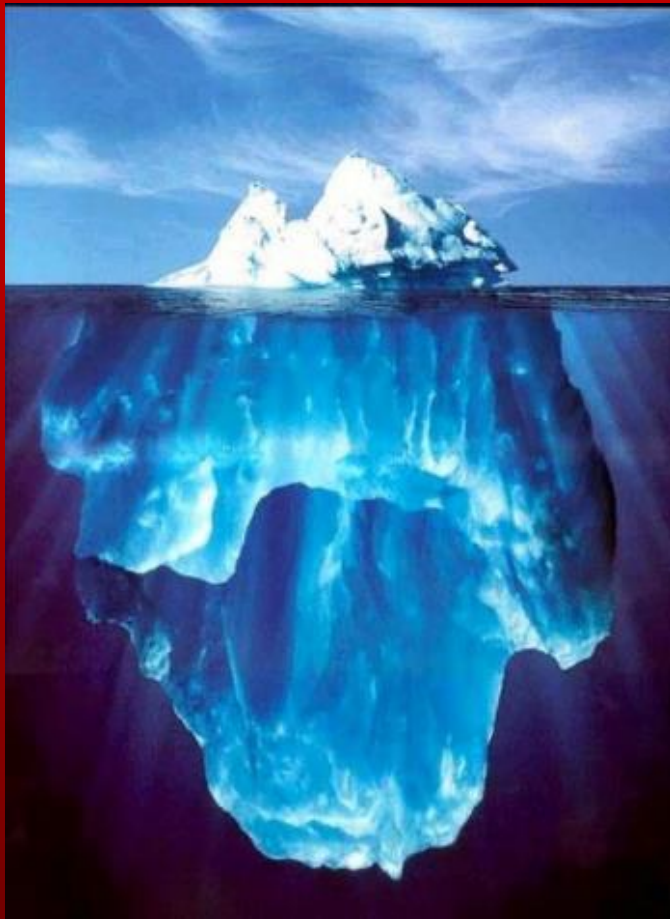
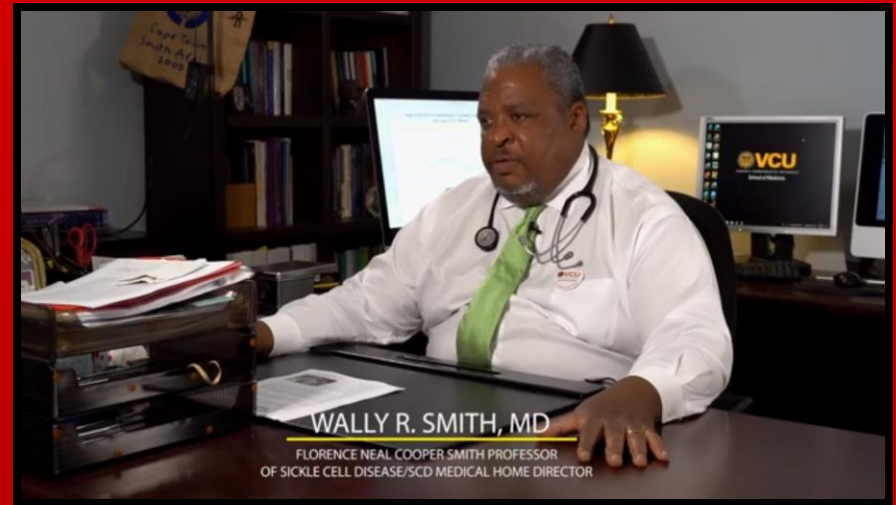


*Photo of Marcus Valentine
from the Chicago Tribune*

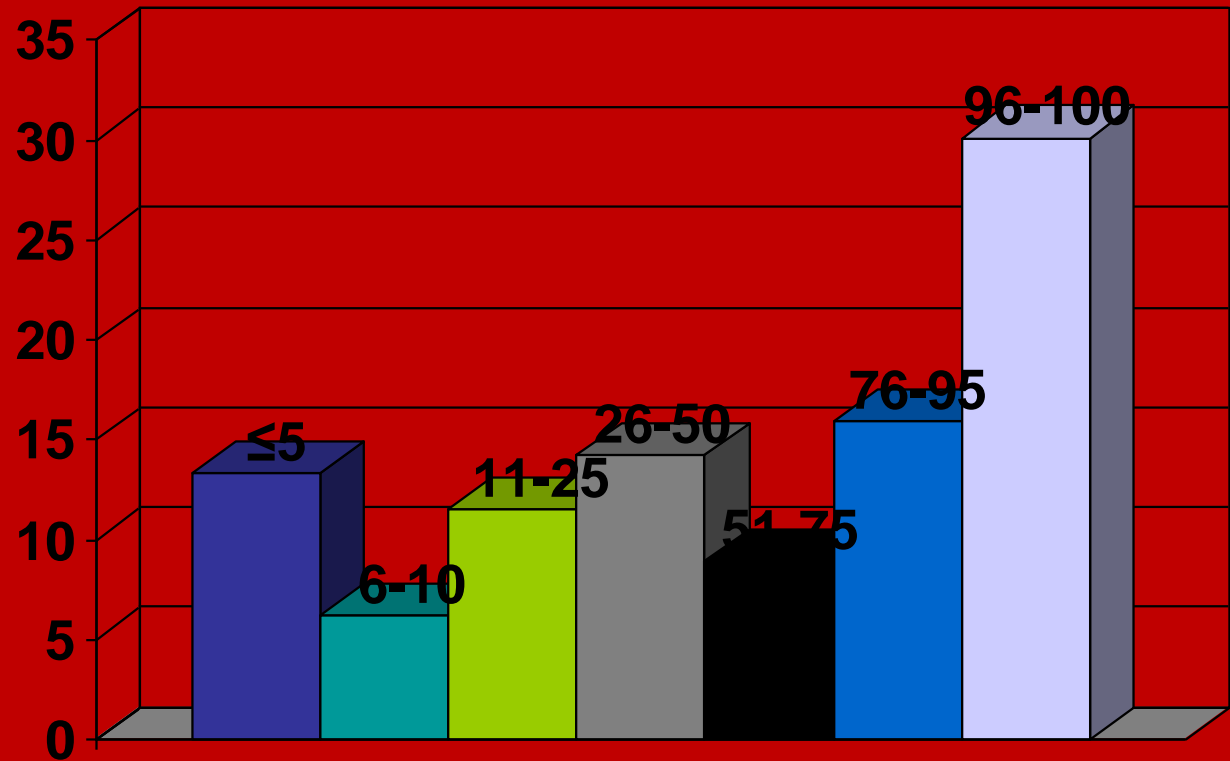


PiSCES Study

Wally Smith



% Pts

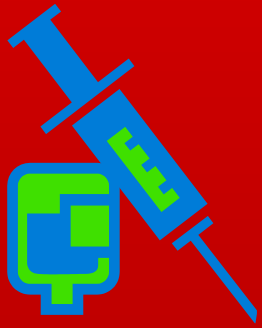


DON'T GO LOOKING FOR SICKLE CELL PAIN...

...IT WILL COME AND FIND YOU

HOW DO I KNOW IF MY BABY IS HAVING
PAIN AND WHAT DO I DO ABOUT IT?





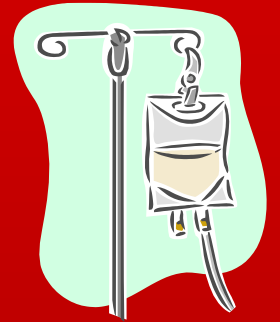
MEDICATION



BED REST
(Limited)



FLUIDS



MANAGING PAIN



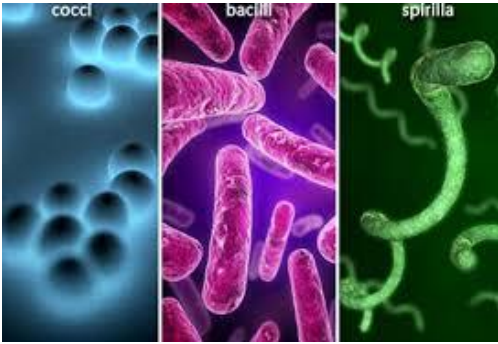
HEAT



MASSAGE



RELAX



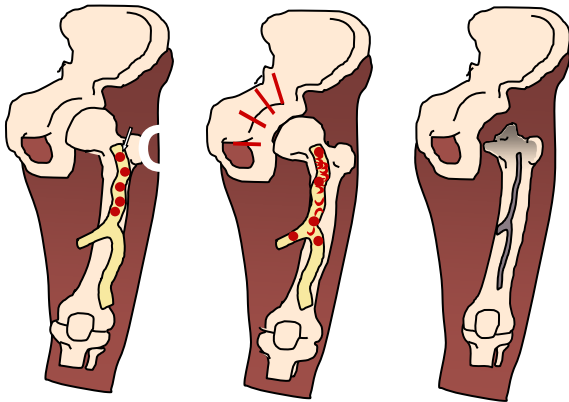
Infection, increased susceptibility to COVID19



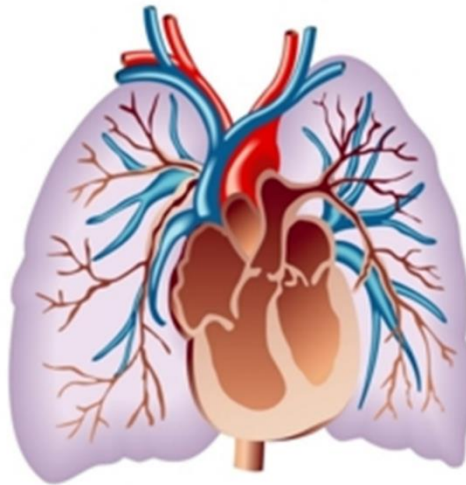
Yellow Jaundice



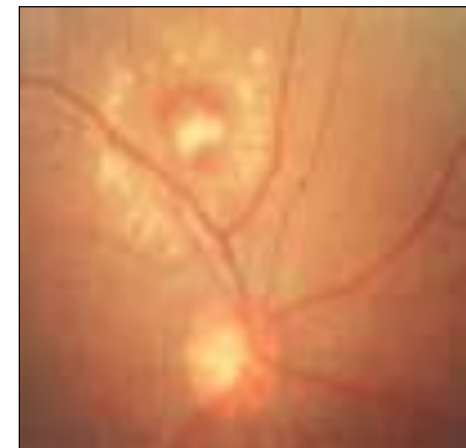
Gallstones



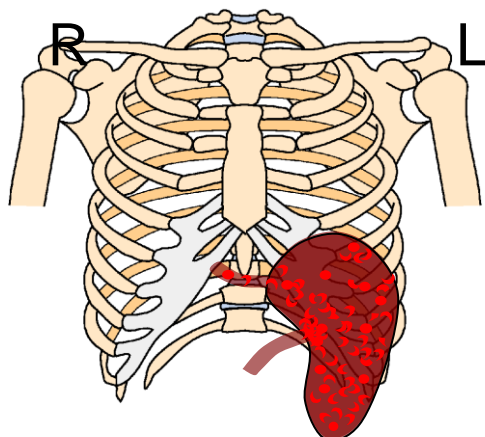
Avascular necrosis AVN



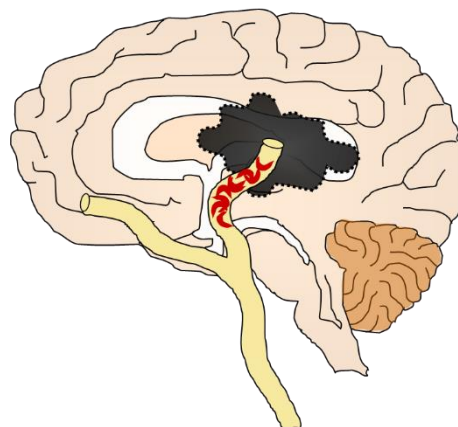
Pulmonary Hypertension



Retinopathy



Splenic sequestration



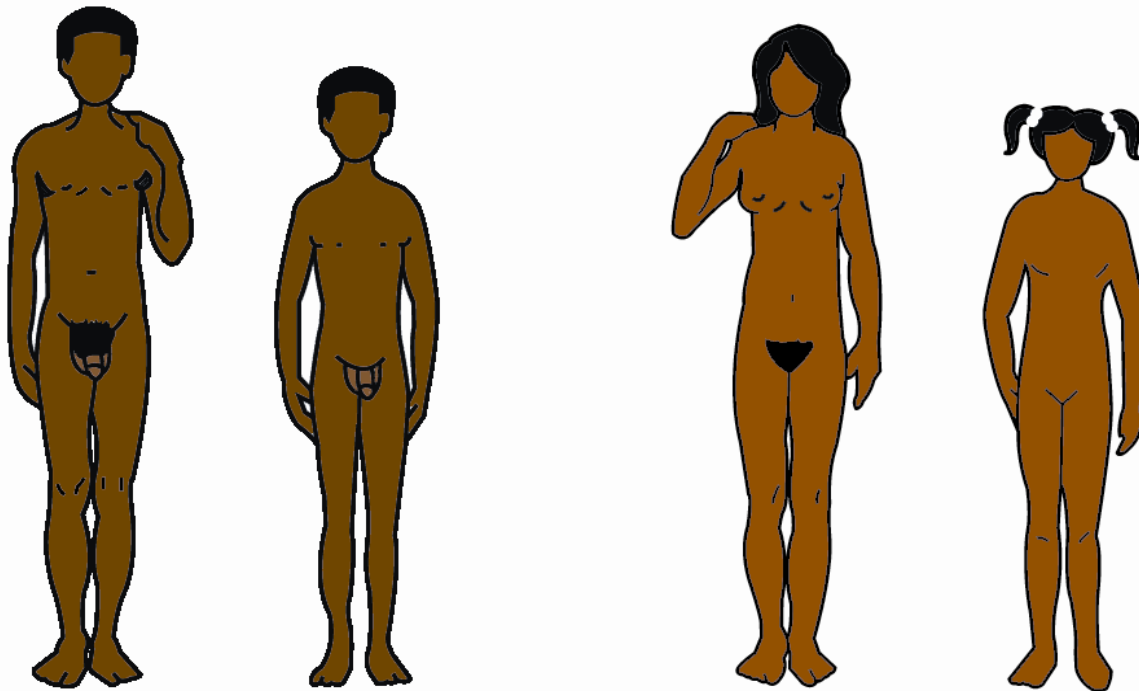
Stroke



Leg Ulcers



Yellow Jaundice

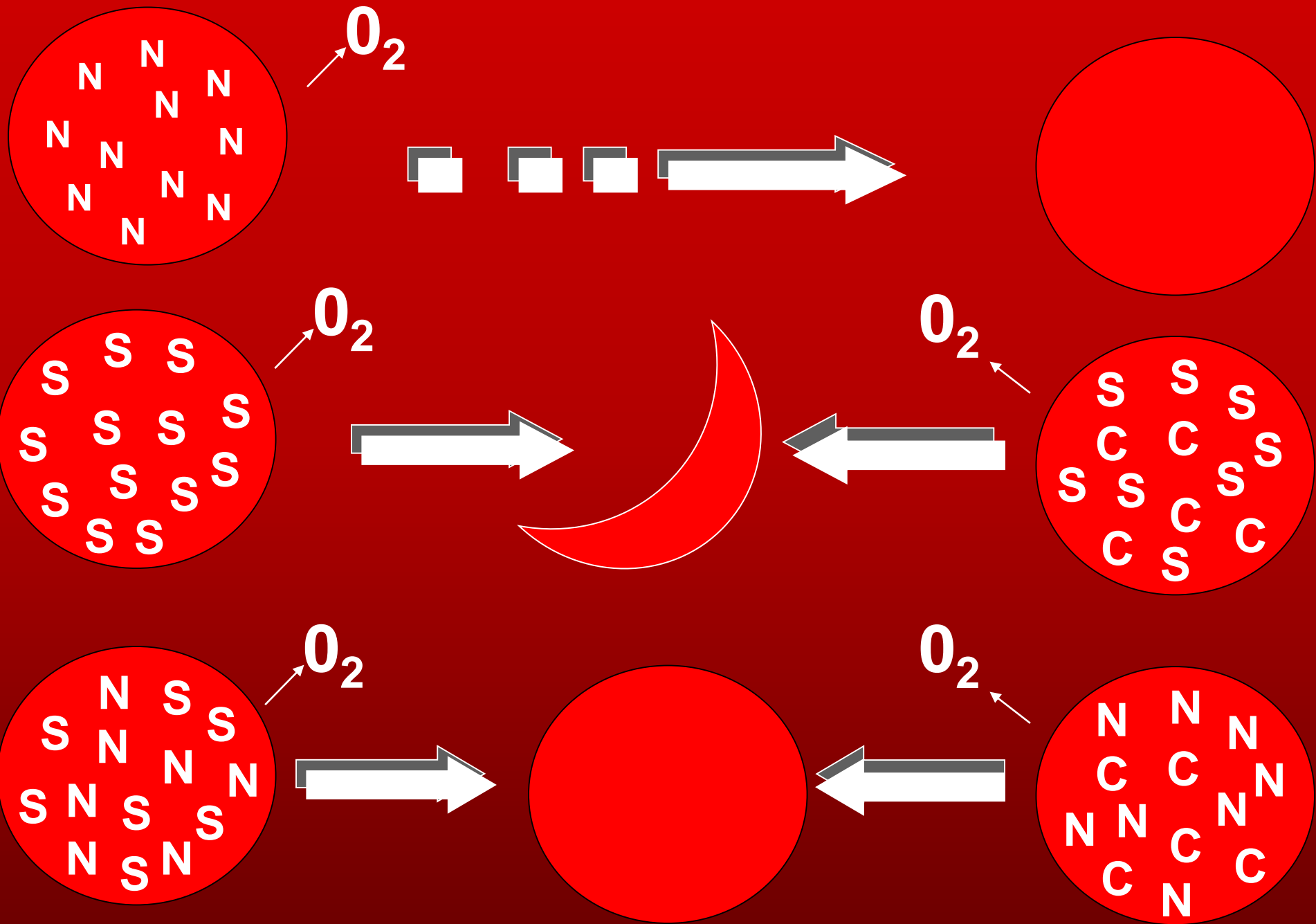


DELAYED GROWTH AND PUBERTY





SCT vs SCD



**Sickle Cell Trait
and
Hemoglobin C Trait
are **NOT**
*DISEASES!***

What you should know if you have SCT:

- ***It is possible for you to have a baby with SCD!***
- SCT does not cause pain and anemia
- You should stay well hydrated
- You should be conditioned for exercise
- Rare problems at high altitudes
- Possibility of blood in urine (painless hematuria) uncommon
- Renal medullary carcinoma
- Hyphema

**UNIVERSAL
PRECAUTIONS**

**No
Children**



Adoption

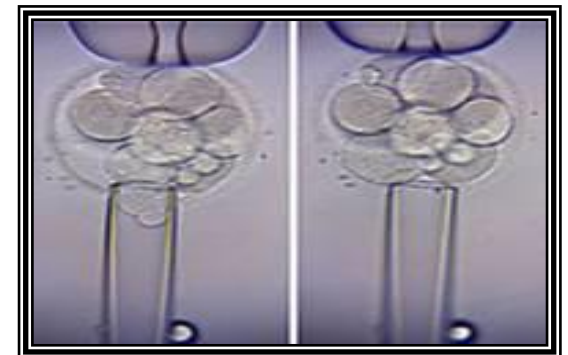
OPTIONS

Prenatal diagnosis



**Amniocentesis
CVS**

**Preimplantation
genetic
diagnosis**



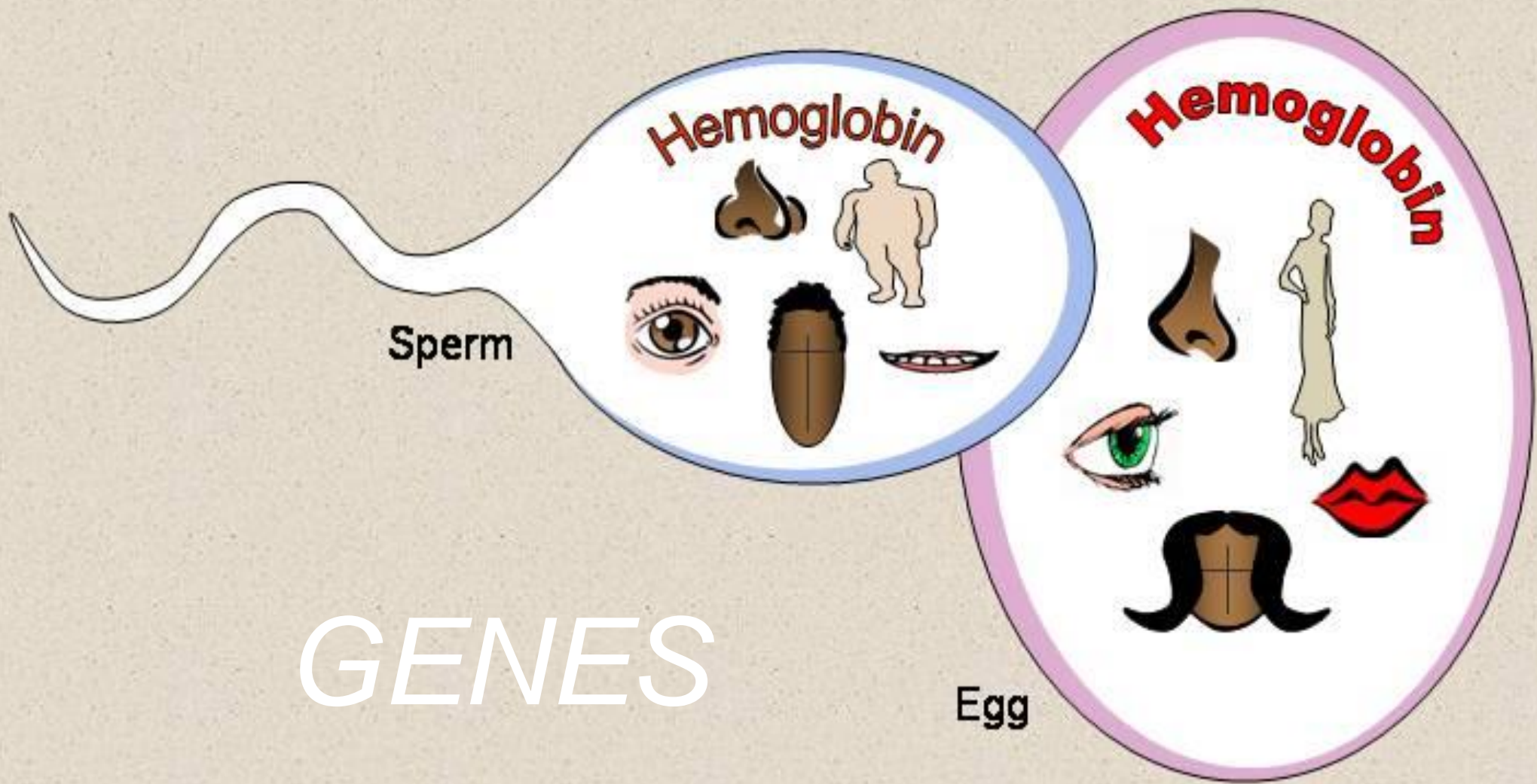
GET YOUR PARTNER TESTED!

- ✓ Sickle Cell Trait
- ✓ Hemoglobin C Trait
- ✓ Thalassemia Trait



HOW DO YOU GET SCD

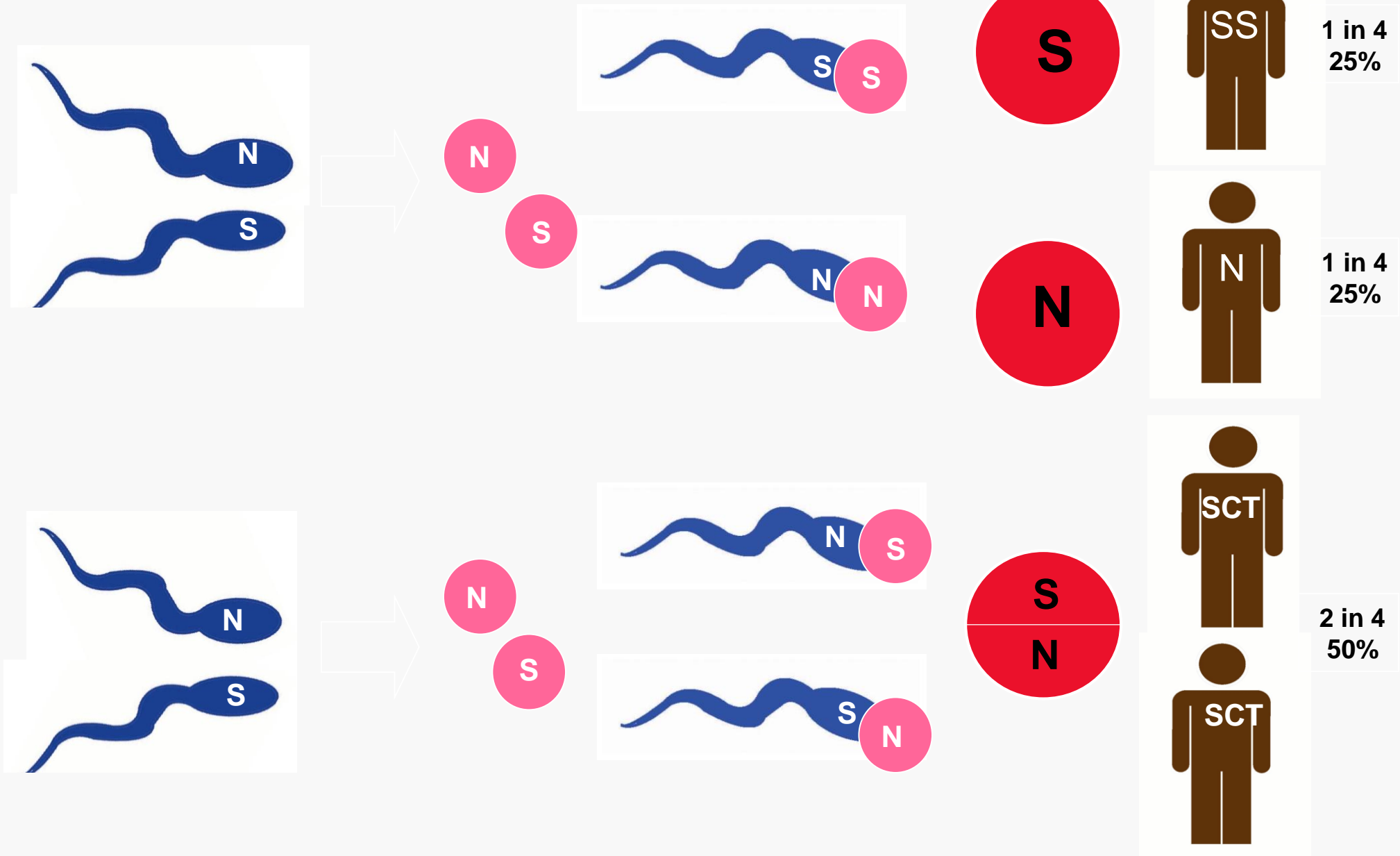
Inherited Characteristics



**Father
SICKLE CELL
TRAIT**

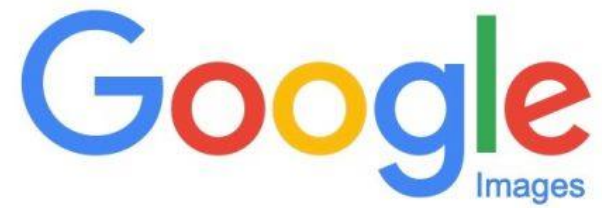
**Mother
SICKLE CELL
TRAIT**

Children



A 3D illustration of a blood vessel, showing a cross-section with a textured, wavy interior wall. Numerous red blood cells, depicted as biconcave discs, are shown flowing through the vessel. The entire scene is bathed in a deep red light, creating a sense of depth and movement. The word "CHALLENGES" is prominently displayed in the center of the image, overlaid on the flow of blood cells.

CHALLENGES

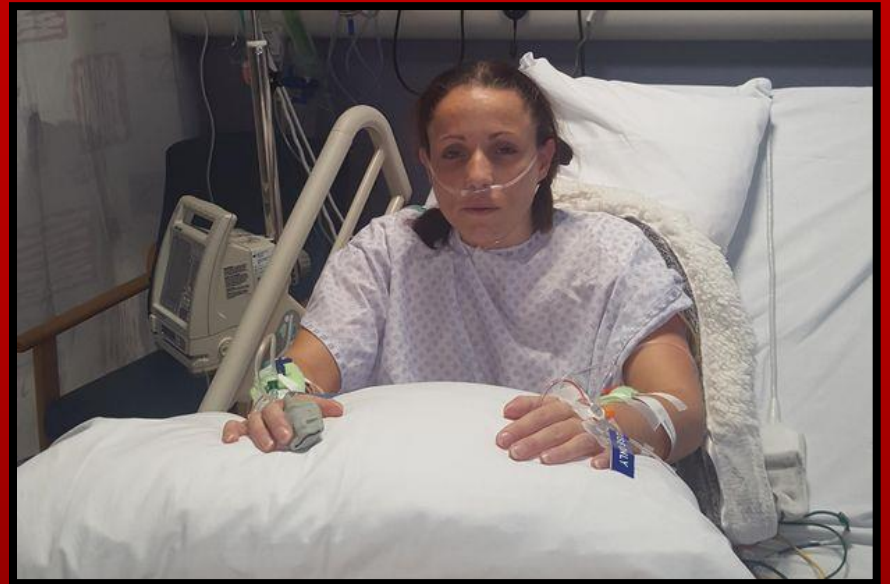


HEALTH CARE DISPARITY





♂ **SCD**



♀ **CF**

NIH Funding and Private Support (Smith, 2006)

Variable	SCD	CF
US prevalence	80,000	30,000
NIH Support, 2004	\$90 mil	\$128 mil
NIH funding/person	\$1,125	\$4,267
# Federal grants		
1968	22	65
1972, after SCA Control Act	215	80
2004	331	459
Private support (2003)		
SCDAA vs CF Found	\$498,577	\$152,231,000
Revenue/person	\$6	\$5,074
Total NIH/private funding	\$90.4 mil	\$280.2 mil
Total support per person	\$1,130	\$9,340



Original Investigation | Health Policy

Comparison of US Federal and Foundation Funding of Research for Sickle Cell Disease and Cystic Fibrosis and Factors Associated With Research Productivity

Faheem Farooq, MD; Peter J. Mogayzel, MD, PhD; Sophie Lanzkron, MD; Carlton Haywood, PhD; John J. Strouse, MD, PhD

January 1, 2008 – December 31, 2018

	CF	SCD
Federal funding per person	\$2807	\$812
Foundation funding per person	\$7690	\$102
Research Articles	1594	926
New FDA approved medications	4	1

Conclusions

The findings show that disparities in funding between SCD and CF may be associated with decreased research productivity and novel drug development for SCD. Increased federal and foundation funding is needed for SCD and other diseases that disproportionately affect economically disadvantaged groups to address health care disparities.

CHALLENGES PATIENTS FACE

- Young people don't know their trait status
- For 20 years only one disease modifying medication
- No universal cure
- Pediatric vs adult care “sickler”
“drug seeker”
- Limited mental health resources



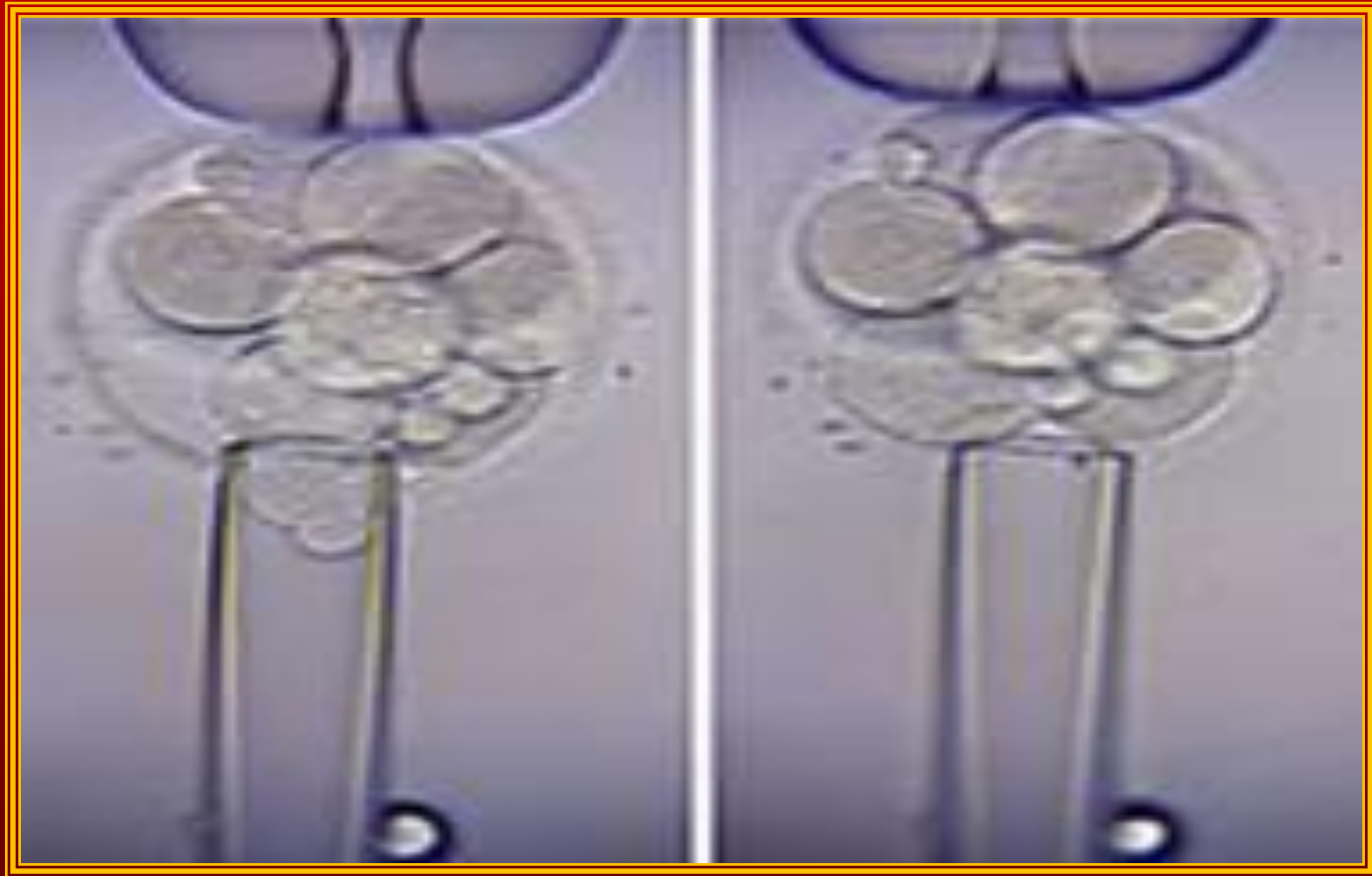
GOOD NEWS

PRENATAL DIAGNOSIS



Amniocentesis
Chorionic Villous Sampling (CVS)

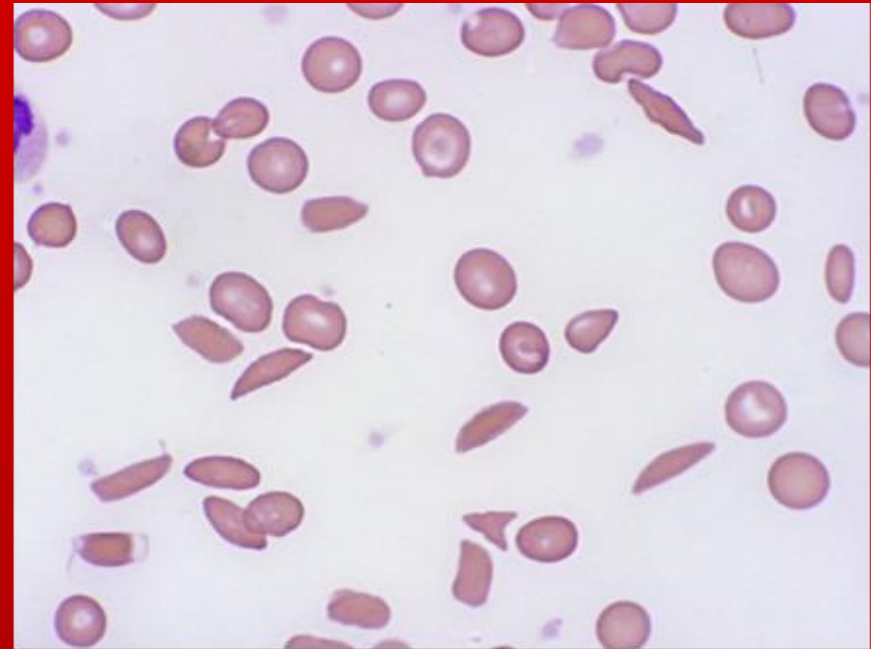
PREIMPLANTATION GENETIC DIAGNOSIS



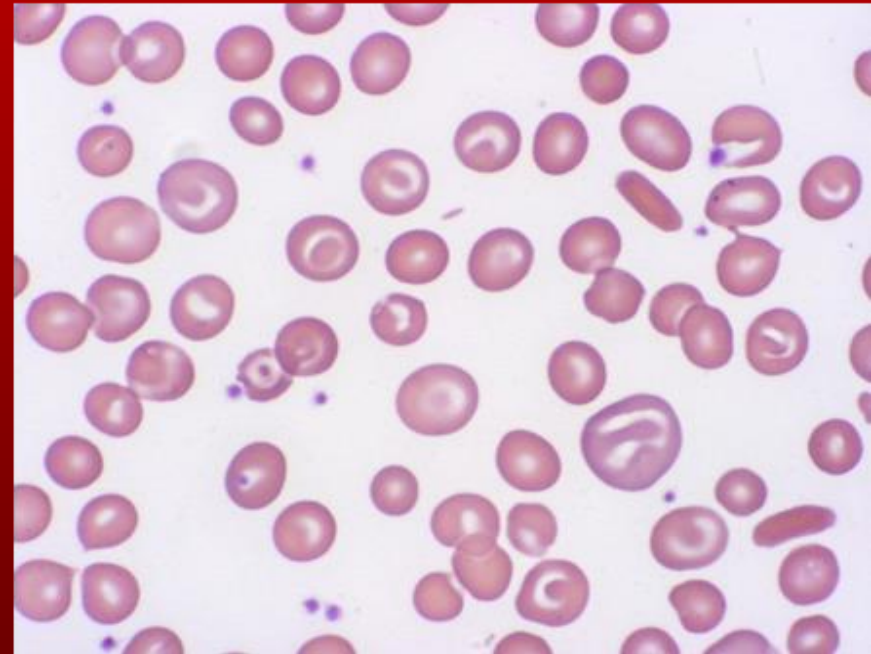


Hydrea, Droxia, Sicklos

Before
Hydroxyurea



Taking
Hydroxyurea



STOP TRIAL



Bob Adams

TCD

11% → 1%

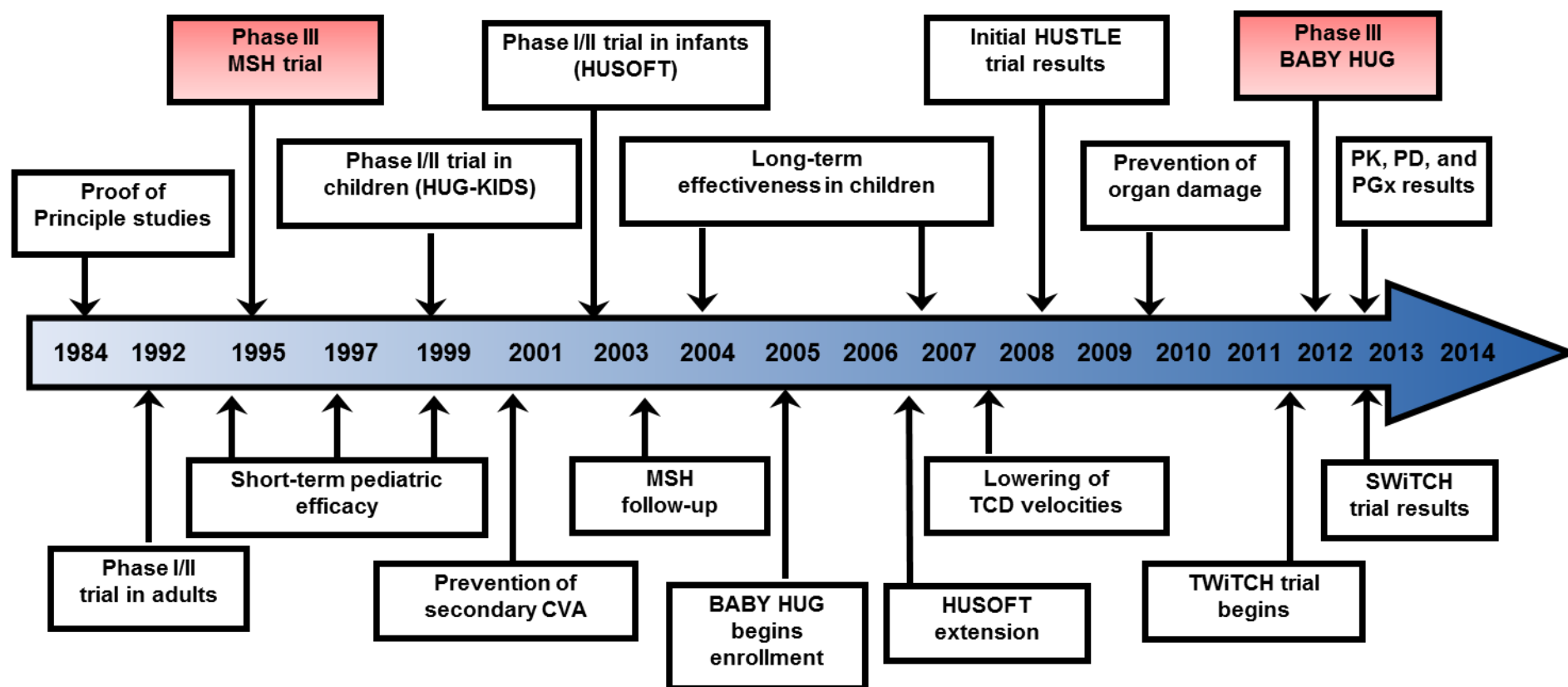
PREVENTION OF A FIRST STROKE BY TRANSFUSIONS IN CHILDREN WITH SICKLE CELL ANEMIA AND ABNORMAL RESULTS ON TRANSCRANIAL DOPPLER ULTRASONOGRAPHY

ROBERT J. ADAMS, M.D., VIRGIL C. MCKIE, M.D., LEWIS HSU, M.D., PH.D., BEATRICE FILES, M.D., ELLIOTT VICHINSKY, M.D., CHARLES PEGELOW, M.D., MIGUEL ABBoud, M.D., DIANNE GALLAGHER, M.S., ABDULLAH KUTLAR, M.D., FENWICK T. NICHOLS, M.D., DUANE R. BONDS, M.D., AND DONALD BRAMBILLA, PH.D.



PRIMARY STROKE PREVENTION

Hydroxyurea: 30 Years of Research



How does it help?

1. Decreases pain episodes
2. Decreases acute chest syndrome
3. Decreases the need for transfusions
4. **People who take it live longer!**



- Start at 9 months
- SS and Sbthal0
- Liquid preparation

YouTube..... SCDAAAMI

July 2017



Oral - Powder
Amino Acid
5 and older
Decreases oxidative Stress

Nov. 2019

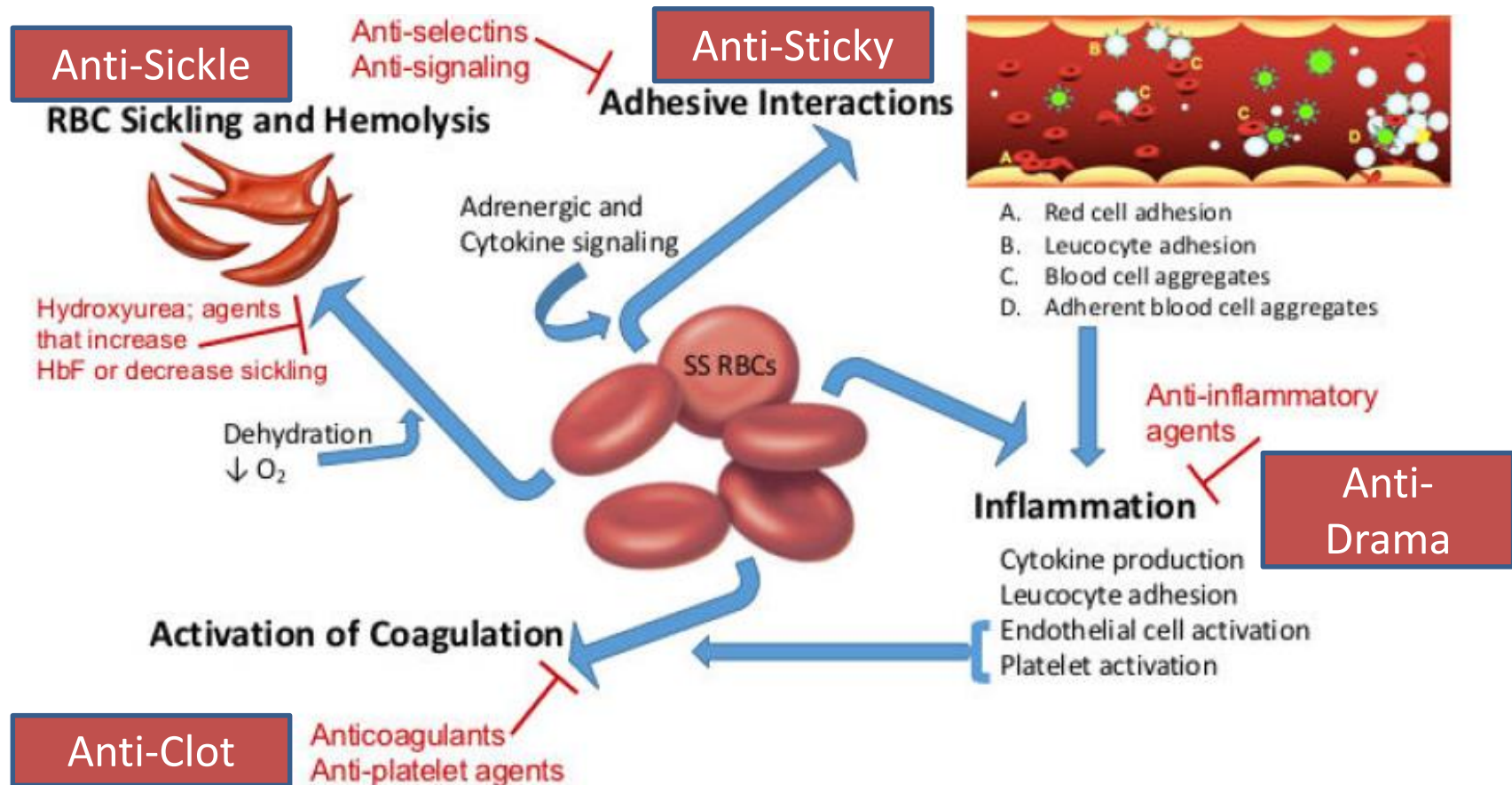


Oral
Small molecule
5 years and older
Increases oxygen affinity

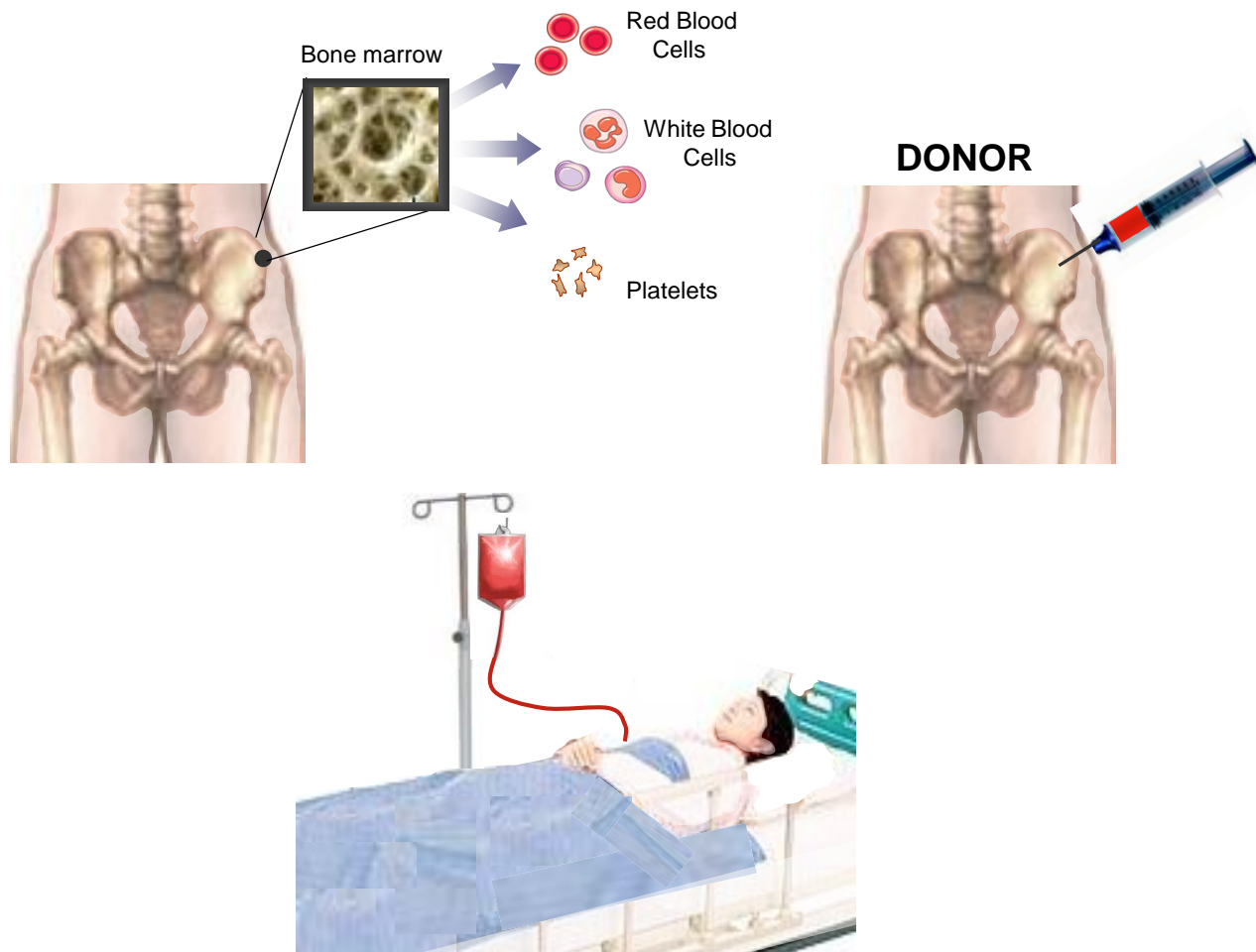


IV
Monoclonal antibody
16 and older
Decreases adhesion

Sickle Cell Disease-Modifying Targets

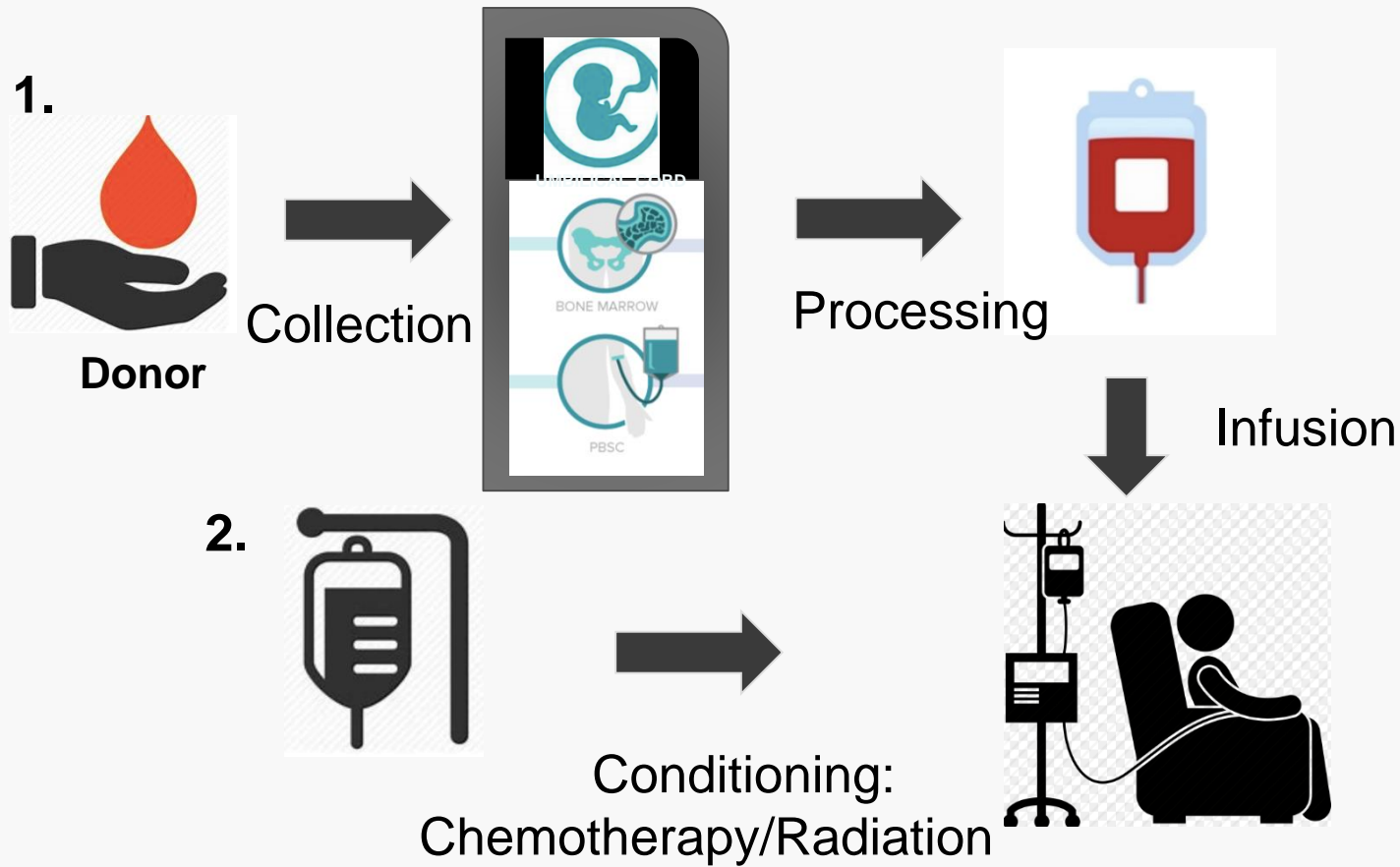


Telen, 2017



BONE MARROW TRANSPLANT (STEM CELL TRANSPLANT)

STEM CELL (BONE MARROW) TRANSPLANT



Courtesy of: Dr. Tami John



CORD BLOOD STORAGE



GENETIC CURE FOR SCD IN THE NEXT 5 YEARS!

**CURE
SICKLE
CELL.**

Gene Therapy & Gene Editing



GENE THERAPY



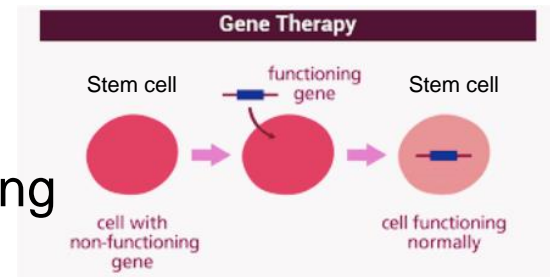
Self



Collection



Processing



2.

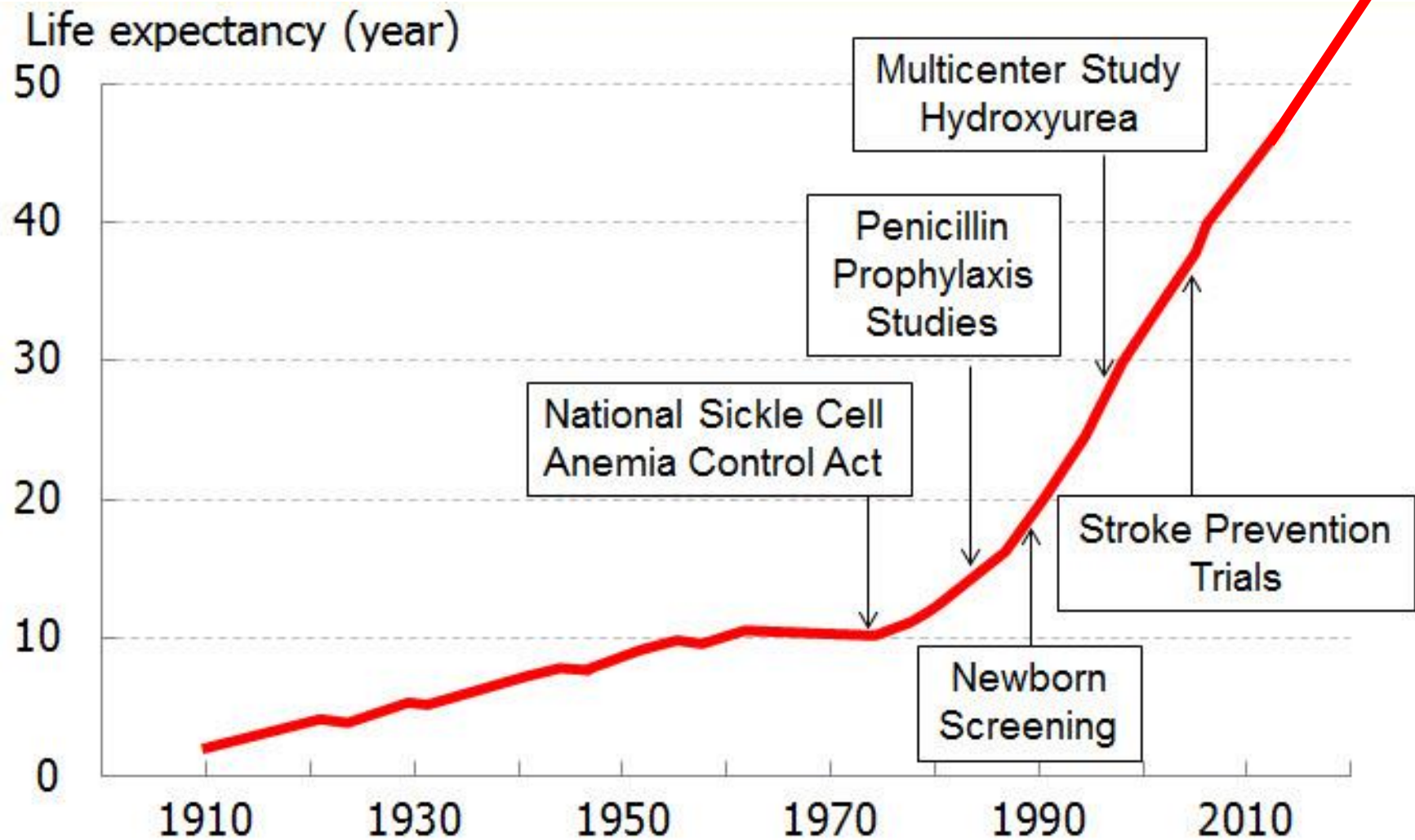


Conditioning:
Chemotherapy

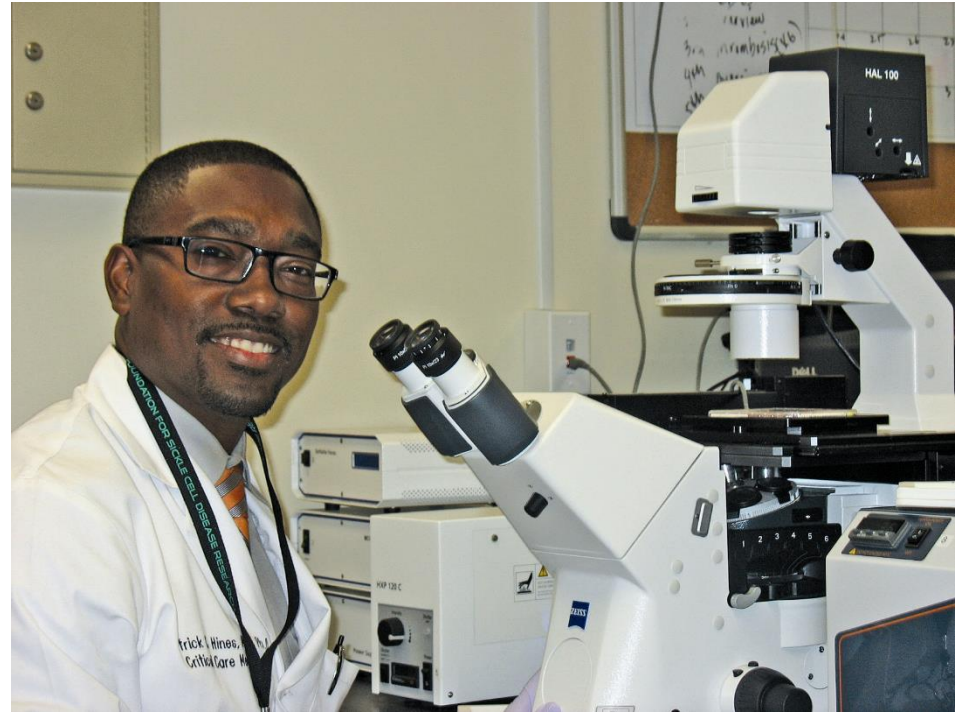


Infusion

Autologous

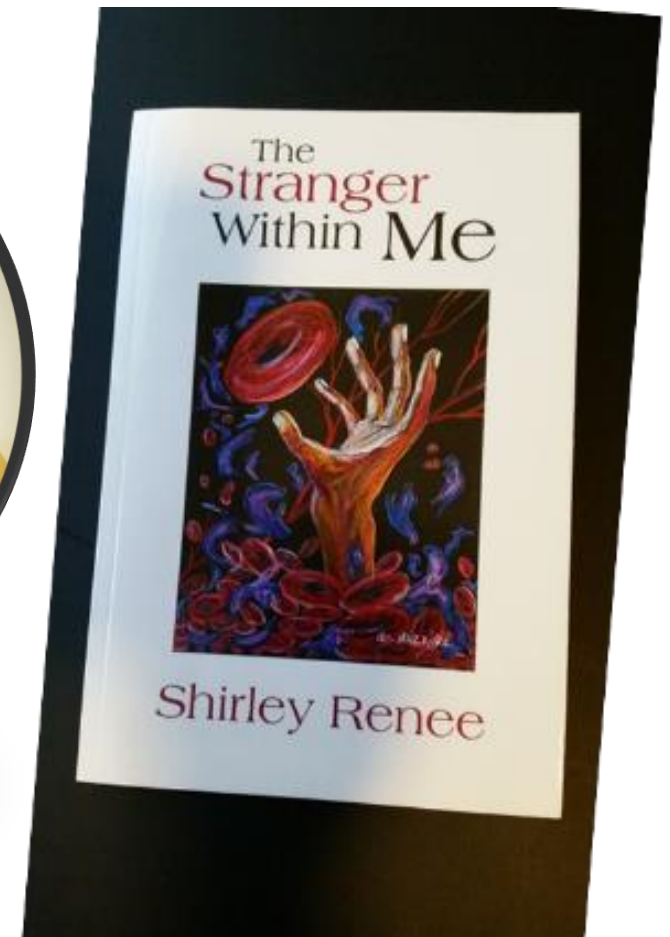
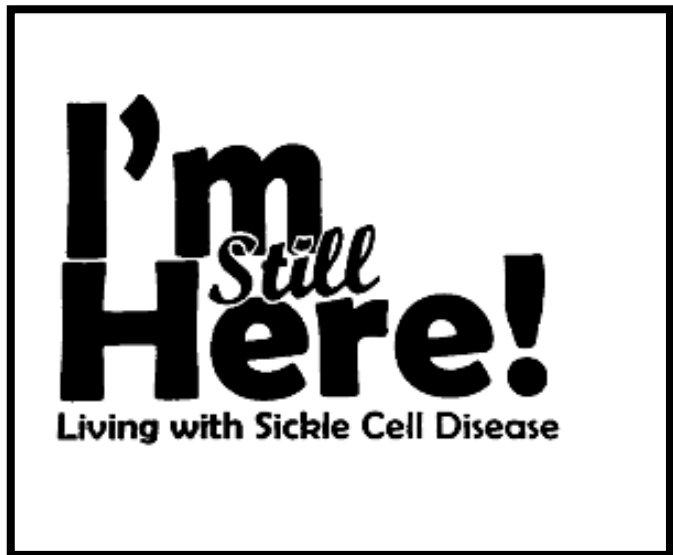


Quality of Life



WARRIORS

Shirley Miller



**Manager - Adult sickle cell program
Carolinas Healthcare System**



“Nothing for us without us”



Dr. Lakiea Bailey

PhD Molecular Hematology



Sickle Cell Consortium and REDHhoTT Present:
The Warrior University Online Series

Blood Transfusions: What You Need to Know and Do
Join us for an online presentation followed by a conversation with leading experts.

To learn more about Warrior University and to register for the free online series, visit
<https://sicklecellconsortium.org/warrioruniversity>
All sessions will begin at 6:30 p.m.

February 25 Blood Transfusions: How, Why, and When?
February 27 Keeping it Safe: Blood Matching
March 4 Keeping it Safe: Iron Overload
March 6 Session 4: African-American Blood Donations: Why are they important?
March 11 Session 5: Let's Talk: Patient-Provider Communication

Presenters include:
James Eckman, M.D., Emory University
Raymona Lawrence, D.P.H., Georgia Southern University
Ashley Singleton, M.P.H., Georgia Health Policy Center

REDHhoTT is the Registry and Education for Hemoglobinopathies and Hemovigilance/Transfusion Therapy in Georgia



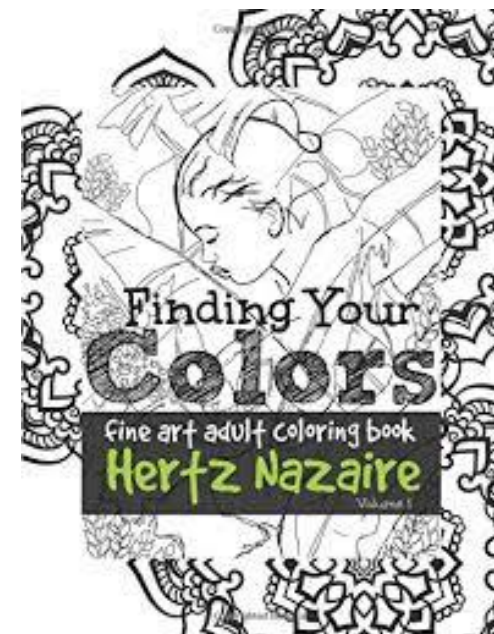
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THE SICKLE CELL REPRODUCTIVE
HEALTH EDUCATION DIRECTIVE



Teonna Woolford

Hertz Nazaire





Sickle Cell Disease Initiative

Component	Description	Cost
CSHCS Adult Benefit Expansion	Extends coverage of SCD treatments to adults 21+ who are not eligible for Medicaid.	\$5,350,000
Enhanced System of Care through Community-Based Organizations	Enhances and expands services provided by the Sickle Cell Disease Association of America to improving the quality of life.	\$690,000
Expanded Clinical Services	Invests in statewide clinical treatment and testing capacity. Expands access to multidisciplinary health care providers.	\$610,000
Total		\$6,650,000

☐ Questions

☐ Comments

☐ Concerns

☐ Suggestions

